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6 MAINTAINING AMERICA'S LEADERSHIP IN BIOMEDICAL INNOVATION:

7 FDA'S ROLE IN ADVANCING U.S. DRUG DEVELOPMENT

8 WEDNESDAY, JULY 15, 2026

9 House of Representatives,

10 Subcommittee on Health,

11 Committee on Energy and Commerce,

12 Washington, D.C.

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16 The subcommittee met, pursuant to call, at 10:28 a.m. in
17 Room 2123 of the Rayburn House Office Building, Hon. Morgan
18 Griffith [chairman of the subcommittee] presiding.

19 Present: Representatives Griffith, Harshbarger,
20 Bilirakis, Carter of Georgia, Joyce, Balderson, Miller-Meeks,
21 Cammack, Obernolte, Langworthy, Kean, and Guthrie (ex
22 officio); DeGette, Ruiz, Dingell, Kelly, Barragan, Schrier,
23 Trahan, Veasey, Fletcher, Ocasio-Cortez, Auchincloss, Carter
24 of Louisiana, Landsman, and Pallone (ex officio)

25 Also present: Representative Mullin.

26

27 Staff Present: Ansley Boylan, Director of Operations;

28 Jessica Donlon, General Counsel; Sydney Greene, Director of
29 Finance and Logistics; Jay Gulshen, Chief Counsel; Annabelle
30 Huffman, Policy Analyst; Megan Jackson, Staff Director;
31 Olivia Jensen, Staff Assistant; AT Johnson, Special Advisor;
32 Daniel Kelly, Press Secretary; Brayden Lacefield, Special
33 Assistant; Madeline Martinez, Staff Assistant; Joel Miller,
34 Deputy Staff Director; Ryan Murphy, Director of Member
35 Services; Brice Ogle, Special Assistant; Clare Paoletta,
36 Professional Staff Member; Claire Richey, Clerk; Seth
37 Ricketts, Clerk; Jake Riith, Staff Assistant; Chris Sarley,
38 Senior Director of Policy and Stakeholder Engagement; Emma
39 Schultheis, Professional Staff Member; Timothy Trimble, Staff
40 Assistant; Matt VanHyfte, Communications Director; Katie
41 West, Press Secretary; Lydia Abma, Minority Policy Analyst;
42 Jordan Antwi, Minority Intern; Shana Beavin, Minority
43 Professional Staff Member; Ruth Campos, Minority Intern;
44 Keegan Cardman, Minority Staff Assistant; Waverly Gordon,
45 Minority Deputy Staff Director and General Counsel; Tiffany
46 Guarascio, Minority Staff Director; Perry Hamilton, Minority
47 Deputy Director, Member Services, and Outreach; Saha
48 Khaterzai, Minority Professional Staff Member; Alyssa Kimura,
49 Minority Intern; Serena Klebba, Minority Intern; Una Lee,
50 Minority Chief Counsel, Health; Brendan Lopez, Minority Press
51 Assistant; Andrew Souvall, Minority Director of
52 Communications, Outreach, and Member Services; Hannah Treger,

53 Minority Staff Assistant; and C.J. Young, Minority Deputy
54 Communications Director.

55

56 *Mr. Griffith. The subcommittee will come to order.
57 The chair recognizes himself for five minutes for an opening
58 statement.

59 Today's hearing will examine ways to maintain American
60 leadership in biomedical innovation. This hearing is a great
61 opportunity to learn more about what is working, where
62 improvements can be made, and how Congress can ensure FDA
63 continues to serve patients well, promote innovation, and
64 maintain our nation's leadership in biomedicine.

65 The United States has always been the global leader in
66 biopharmaceutical research and development, and it is
67 important that we remain leaders in this space. However,
68 other countries are catching up to the United States. It is
69 important for Congress to reexamine our current landscape to
70 ensure we are staying up to date with the latest
71 biopharmaceutical advancements and examine what role Congress
72 could play in making these processes more efficient. This
73 Administration has proposed efforts to do so, such as the
74 recent launch of FDA's Operation Trailblazer -- or
75 Trialblazer, excuse me -- announced in June. This is -- you
76 know, you rehearse this stuff, you still get it wrong.

77 Trialblazer was announced in June. This is an HHS-wide
78 effort that comes in response to the growing competitiveness
79 of the global pharmaceutical landscape which promotes cross-
80 agency collaboration in support of research from the earliest

81 stages of innovation. Operation Trialblazer provides a set
82 of initiatives and reforms to modernize and accelerate
83 medical product development, aiming to remove the unnecessary
84 hurdles and ambiguity that sometimes hinders patient access
85 to innovative treatments. Streamlining clinical trial
86 requirements, encouraging use of modern trial designs and real-
world
87 evidence where appropriate, and reducing administrative burden
88 can shorten development timelines and lower costs without
89 compromising science or safety. These improvements have the
90 potential to bring new treatments to patients faster,
91 increase participation in clinical research, and make the
92 United States even more attractive for investment in medical
93 breakthroughs.

94 It is important that we begin to have these
95 conversations as this subcommittee looks ahead to FDA's user
96 fee reauthorizations next year, especially as initiatives
97 like Operation Trialblazer demonstrate that an approach to
98 maintaining America's competitive edge involves improving
99 the efficiency and efficacy of drug development.

100 For more than three decades, user fee programs have
101 helped provide FDA with the resources to review innovative
102 drugs and medical devices while maintaining the agency's
103 rigorous standards for safety and effectiveness. Rather than
104 relying solely on taxpayer dollars, user fees are paid by

105 manufacturers that submit products for review, helping ensure
106 the FDA has the scientific expertise and capacities necessary
107 to keep pace with the rapidly advancing medical innovations.
108 Ultimately, user fees serve to improve health outcomes for
109 patients. Every day that a safe and effective therapy
110 reaches the market is another day that patients living with
111 cancer, rare diseases, or other serious conditions may have
112 access to life-changing or even lifesaving treatments.

113 The predictability and performance goals established
114 through the user fee agreements have helped reduce review
115 times, improve communication between FDA and product
116 developers, and provide greater standardization through the
117 review process. However, there is still more work that can
118 be done, and reauthorization is not limited to just extending
119 the status quo. Reauthorization gives Congress the
120 opportunity to explore and write legislation so we can
121 continue to work to streamline regulatory processes, reduce
122 unnecessary administrative burdens, strengthen communication
123 with product developers, and ensure the agency has the tools
124 it needs to make the best possible use of its resources.

125 We can also continue to look for opportunities to
126 modernize clinical trial networks, embedding them in routine
127 care and expanding access to innovative treatments in rural
128 areas. Maintaining America's position as the global leader
129 in biomedical innovation requires a regulatory system that is

130 both thorough and efficient. I am eager to look for ways
131 that we can help ensure FDA can review drugs in a timely and
132 predictable manner not only to expand patient access to
133 innovative treatments, but to also encourage companies to
134 invest, develop, and manufacture domestically.

135 I thank the witnesses for being here this morning and I
136 look forward to the discussion.

137 [The prepared statement of Mr. Griffith follows:]

139

140 *****COMMITTEE INSERT*****

141

142 *Mr. Griffith. I now recognize the subcommittee ranking
143 member, Ms. DeGette, for her five minutes for an opening
144 statement.

145 *Ms. DeGette. Thank you so much, Mr. Chairman. I am
146 really excited about this hearing today because I have really
147 worked a lot on biomedical research issues over my career.
148 And I have to tell you I think that there is -- I have never
149 seen a more precarious time for the U.S. leadership around
150 the world in biomedical research.

151 The FDA plays a critical role in regulating clinical
152 trials centering on the safety of trial participants and
153 making sure that trials are asking the right questions. Over
154 the past year and a half, FDA's staff has been decimated.
155 Political appointees have been making decisions that should
156 be left to scientists, and industry and researchers have been
157 left in the dark about what current agency policy actually
158 is. The disastrous reign of Elon Musk and DOGE last year
159 resulted in 3,500 jobs being eliminated at the FDA willy
160 nilly, without respect to what they were, the most of any HHS
161 agency. Low morale was pervasive at the agency, and it
162 showed. FDA shed senior leaders and rank-and-file scientists
163 alike, even the ones who weren't just summarily dismissed.

164 Under Commissioner Makary an agency famously insulated
165 from political pressures became instead captured by them. He
166 started the Commissioners National Priority Voucher program,

167 which provided incentives to companies with no pre-defined
168 criteria, simply rewarding general alignment with
169 Administration policies. Without set public criteria,
170 companies don't know what to focus on for the program and the
171 public doesn't know what legitimate or not is being rewarded.

172 In another example, bowing to Secretary Kennedy's anti-
173 vaccine nonsense, FDA Commissioner Makary refused to even
174 review an mRNA-based flu vaccine before being forced to
175 change course by public outcry.

176 And who can forget FDA's initiation of a label change
177 for Tylenol indicating a lack of safety for use in pregnancy,
178 contrary to established scientific evidence and against the
179 advice of medical organizations simply because Secretary
180 Kennedy wanted something to blame for increased documented
181 rates of ADHD and autism?

182 All of these decisions placed ideology above science.
183 FDA's inability and shoot-from-the-hip approach to policy
184 also impacted industry. Commissioner Makary and FDA
185 leadership made major policy announcements through journal
186 articles, press releases, and even podcasts, circumventing
187 FDA's policy-making processes and making it so researchers
188 did not know what the official policy was.

189 FDA would even contradict its own past guidance, as in
190 the case of experimental gene therapy for Huntington's
191 disease. FDA reversed direction, demanding a new trial for

192 the product, even though advocates and scientists believe the
193 new trial to be unethical.

194 Luckily, with the evidence that Makary is gone, things
195 do seem to be slowly improving, and this is what I am hearing
196 from the community. The agency is now in the process of
197 hiring thousands of staff to fill critical gaps, though it is
198 hindered by the reputational damage to the agency from Makary
199 and DOGE. There are responsible leaders atop the critical
200 centers at FDA, and Acting Commissioner Kyle Diamantis has
201 shown every indication that he will empower career experts to
202 do their jobs and protect American patients and get good
203 markets to -- products to market.

204 In February I sent a letter to FDA regarding the
205 agency's failure to follow its own good guidance practices,
206 announcing policy through journal articles, et cetera, et
207 cetera, which is expressly prohibited in regulation. Last
208 week the agency finally responded, indicating the
209 announcements we asked about were not, in fact, guidance and
210 did not communicate FDA policy on these issues.

211 I would like to enter both my letter and the agency's
212 response in the record. I would like to ask unanimous
213 consent, Mr. Chairman.

214 *Mr. Griffith. Without objection, so ordered.

215 [The information follows:]

216

217 *****COMMITTEE INSERT*****

218

219 *Ms. DeGette. I hope this is a turning point back to
220 transparency, to consistency, and to a focus on good science.
221 This is particularly important for developers trying to get
222 promising medicines into the clinic, and it is also important
223 to our reputation in the world so we can get clinical trials
224 back into the U.S. and stop this brain drain that we have
225 seen going overseas. Innovative researchers will need to
226 trust the FDA to treat their applications fairly and without
227 political favor or disfavor.

228 As science -- as our capabilities evolve, so must our
229 regulators. And so, needless to say, Mr. Chairman, I am
230 looking forward to hearing more from the witnesses on just
231 how we can work with the agency to strengthen it so that we
232 can get cures to patients as quickly and safely as possible,
233 and maintain our leadership in biotechnology and medicine as
234 we go through the 21st century.

235 And I yield back.

236 *Mr. Griffith. The gentleman yields back. I now
237 recognize the chairman of the full committee, Chairman
238 Guthrie, for five minutes for his opening statement.

239 *The Chair. Good morning, and thank you, Chairman
240 Griffith, for holding this hearing on this important topic.
241 And the Ranking Member DeGette, thank you for doing this.
242 Additionally, thank you to all of our witnesses for being
243 here. We appreciate your expertise as we examine how

244 Congress and the FDA can strengthen America's leadership in
245 biomedical innovation by supporting early-stage drug
246 development, clinical research, and domestic manufacturing.

247 For decades the United States has set the global
248 standard, a gold standard for developing safe and effective
249 medical products which benefit patients around the world with
250 Americans who gain earlier access to ground-breaking
251 therapies [sic]. This is possible because innovators have
252 chosen to develop them here first.

253 But our leadership cannot be taken for granted. Other
254 countries, particularly China, are making significant
255 investments to attract biotechnology companies, their
256 clinical trials, and domestic manufacturing. Our ability to
257 develop and manufacture lifesaving medicines, vaccines, and
258 other medical products here in the U.S. depends on a strong
259 domestic biotechnology sector and a regulatory environment
260 that supports innovation while preserving FDA standards for
261 safety and effectiveness.

262 Bringing new therapies to patients requires clinical
263 trials that are efficient and modern, leveraging new digital
264 technologies and novel evidence supported by sound science.
265 This committee has a strong record of advancing those goals.
266 Through the FDA user fee authorization this committee has
267 worked in a bipartisan way to modernize FDA's authorities,
268 improve regulatory predictability, and help Americans access

269 new cures without compromising FDA's gold standard of review.
270 Today's hearing is an opportunity to continue that work,
271 learning from our witnesses where barriers to innovation
272 remain, how FDA can further improve the efficiency and
273 predictability of early-stage drug development, and what
274 legislative changes Congress should consider as we prepare
275 for the next FDA user fee reauthorization cycle.

276 [The prepared statement of The Chair follows:]

277

278 *****COMMITTEE INSERT*****

279

280 *The Chair. Thank you, Mr. Chairman. I appreciate our
281 witnesses for being here, and I yield back.

282 *Mr. Griffith. The gentleman yields back. I now
283 recognize the ranking member of the full committee,
284 Representative Pallone, for his five minutes for an opening
285 statement.

286 *Mr. Pallone. Thank you, Chairman Griffith. I am
287 pleased that we are here to talk about advancing the U.S.'s
288 role in drug development, particularly at a time when that
289 role is threatened by the actions of the Trump
290 Administration.

291 For a long time the U.S. has been the world leader in
292 drug development, but this did not just happen. It was
293 thanks to the cooperation between our public health agencies,
294 namely the Food and Drug Administration and the National
295 Institutes of Health, and industry partners to ensure that
296 our gold standard research and regulatory review process is
297 appropriately tailored to bring safe and effective products
298 to Americans.

299 Other countries have looked to our public health
300 agencies as the gold standard leaders in research,
301 therapeutic innovation, and development, and regulatory
302 oversight of medical product assessment. In order to remain
303 that global leader we need to preserve the strength of our
304 public health infrastructure and investment in research.

305 However, we have heard many experts sound the alarm that the
306 United States is at risk of losing its competitive edge in
307 research and development to other countries, especially
308 China.

309 Unfortunately, actions we have seen from the Trump
310 Administration will only fuel the fire and allowing other
311 countries to overtake our position, threatening the
312 advancement of lifesaving cures. The Trump Administration
313 has slashed billions of dollars in critical research funding.
314 This funding was already congressionally approved, but Trump
315 wasted no time in abruptly and arbitrarily cutting the
316 funding from research institutions and dismantling Federal
317 scientific agencies.

318 Experts warn that these funding cuts destabilize the
319 scientific research that is the foundation of drug discovery.
320 In many instances these funds were slated to go towards
321 scientific research that would develop basic research that
322 complements industries' applied research for drugs that they
323 submit to FDA for approval. Without this initial public
324 investment into NIH, the pipeline of drugs will slow. In
325 fact, the Congressional Budget Office estimated that a 10
326 percent cut to NIH's budget would result in 20 fewer
327 lifesaving drugs approved over 10 years.

328 But the Administration's threats to advancing drug
329 development do not end there. Early last year the Elon Musk-

330 led DOGE indiscriminately and illegally cut thousands of
331 Federal employees from our public health institutions. It is
332 still unclear which positions or programs were cut in the
333 mass layoffs. And I have asked FDA specifically for this
334 information for over a year and still have not received
335 satisfactory responses. Just yesterday I asked the
336 Government Accountability Office whether FDA has violated the
337 law in spending user fee dollars on unauthorized
338 expenditures. And as we approach the user fee
339 reauthorization next year, I expect answers to those
340 questions.

341 Beyond these terminations, the Administration has
342 created upheaval at the FDA. Despite attempts to rehire
343 staff, reports have shown that the FDA applicant pools are
344 not as robust as they used to be. This is concerning, given
345 reports of brain drain with leading scientists leaving the
346 United States to work for competing countries.

347 So I am glad we are having this hearing today, but we
348 cannot have a productive conversation about how best to move
349 forward without honestly evaluating the real harm that the
350 Trump Administration has done. The Administration's actions
351 threaten availability of new cures for American patients, and
352 I look forward to hearing from our witnesses about what else
353 is needed to address this critical problem as we begin the
354 process of reauthorizing user fees.

355 [The prepared statement of Mr. Pallone follows:]

356

357 *****COMMITTEE INSERT*****

358

359 *Mr. Pallone. So thank you, Chairman Griffith, and I
360 yield back.

361 *Mr. Griffith. The gentleman yields back. We now
362 conclude with opening statements. The chair would like to
363 remind members that, pursuant to committee rules, all
364 members' opening statements will be made a part of the
365 record.

366 We want to thank our witnesses for taking their time to
367 testify before the subcommittee today. Although it is not
368 the practice of this subcommittee to swear in witnesses, I
369 would remind our witnesses that knowingly and willfully
370 making materially false statements to the legislative branch
371 is against the law under title 18, section 1001 of the United
372 States Code.

373 You will have the opportunity to give an opening
374 statement, followed by questions from members. And our
375 witnesses today are Dr. Cynthia Verst, who is the president
376 of design and delivery innovation, R&D Solutions at IQVIA;
377 Ms. Susan Winckler, who is the executive officer of the
378 Reagan-Udall Foundation for the FDA. And I will now yield to
379 my friend and colleague, Ms. DeGette of Colorado, to
380 introduce our next witness.

381 *Ms. DeGette. Thank you. Thank you, Mr. Chairman. I
382 am so happy to introduce my friend, Dr. Aaron Kowalski, who
383 is the chief executive officer of Breakthrough T1D that --

384 which is the leading global organization driving research,
385 advocacy, and cures for type 1 diabetes.

386 Many people on this committee know my daughter,
387 Francesca, who was diagnosed with type 1 diabetes very early
388 in my congressional career. And also Dr. Kowalski and his
389 brother both have it. Frankly, when they were all diagnosed,
390 everybody was saying there is a cure around the corner, no
391 problem, and all these years have gone by. But all of us
392 have shared a lifetime commitment to advancing treatments,
393 therapies, and ultimately cures. And it has been
394 extraordinary to see what has transpired.

395 And so, this is why I am so happy Dr. Kowalski is here,
396 and it will blow your mind when he talks to you about some of
397 this research, but also the others here today, what is
398 happening in biomedical research in this country. And I know
399 that under his leadership Breakthrough T1D continues to bring
400 researchers, industry, regulators, and advocates together.

401 And I just want to say, while I have the floor, thank
402 you to Dr. Kowalski, but thank you to all of our witnesses
403 here today and everybody in the room. We are on the cusp of
404 exciting, exciting discoveries, and this committee needs to
405 partner with all of you in how we do it.

406 Okay. I yield.

407 *Mr. Griffith. And I appreciate it, and I also
408 appreciate all the witnesses being here today.

409 The next witness to be introduced is Dr. Thomas Hwang.
410 Did I get it right? All right. He says I got it right. And
411 is an assistant professor of medicine at Brigham and Women's
412 Hospital and Harvard Medical School.

413 And last but not least, Dr. Thomas Bollyky is the
414 Bloomberg chair in global health, senior fellow for
415 international economics, law, and development, and director
416 of the global health program, Council on Foreign Relations.

417 Per committee custom, each witness will have the
418 opportunity for a five-minute opening statement followed by a
419 round of questions from members.

420 The light on the timer in front of you will turn from
421 green to yellow when you have one minute left.

422 It is now my honor and privilege to recognize our first
423 witness, Dr. Cynthia Verst for her five-minute opening
424 statement.

425

426 STATEMENT OF CYNTHIA VERST, PHARMD, MS, PRESIDENT, DESIGN AND
427 DELIVERY INNOVATION, R&D SOLUTIONS, IQVIA; SUSAN C. WINCKLER,
428 RPH, ESQ., CEO, REAGAN-UDALL FOUNDATION FOR THE FDA; AARON J.
429 KOWALSKI, PHD, CEO, BREAKTHROUGH T1D; THOMAS HWANG, MD,
430 ASSISTANT PROFESSOR OF MEDICINE, BRIGHAM AND WOMEN'S
431 HOSPITAL AND HARVARD MEDICAL SCHOOL; THOMAS K. BOLLYKY, JD,
432 BLOOMBERG CHAIR IN GLOBAL HEALTH, SENIOR FELLOW FOR
433 INTERNATIONAL ECONOMICS, LAW, AND DEVELOPMENT, AND DIRECTOR
434 OF THE GLOBAL HEALTH PROGRAM, COUNCIL ON FOREIGN RELATIONS
435

436 STATEMENT OF CYNTHIA VERST

437

438 *Dr. Verst. Chairman Griffith, Ranking Member DeGette,
439 and members of the subcommittee, thank you for the invitation
440 to present to you today. I am Dr. Cindy Verst, president of
441 R&D design and delivery innovation for IQVIA, a leading
442 clinical research service and commercial insight provider to
443 the life science industry.

444 Our 90,000 employees work every day to serve over 1,000
445 biopharmaceutical sponsors, from large to small, bringing
446 medicines to patients faster. We connect globally with
447 thousands of investigators to engage patients and deliver
448 high-quality clinical data to global regulators.

449 IQVIA's vantage point today on the U.S. competitiveness
450 is both practical, pragmatic, and data-driven. We help

451 sponsors design and execute global clinical trials, and we
452 see in real time why companies choose one country over
453 another for their trials. So I would like to share a few
454 insights and data points and recommendations.

455 Firstly, the United States remains the most sought-after
456 market for new medicines, and thus a critical destination for
457 clinical trials. And although our data does confirm that
458 China surpassed the U.S. share of phase one innovative
459 industry trials starts in 2021, at the end of 2025 China
460 accounted for 35 percent and the U.S. 27 percent of phase one
461 trial share. In looking more closely at the data, I would
462 like to share other insights.

463 China's phase one growth is fueled by Chinese-
464 headquartered sponsors -- that is, by China for China -- and
465 where their home-grown information relative to innovation is
466 translating at scale from bench to the clinic. In fact, 89
467 percent of phase one trial country uses by Chinese companies
468 in 2025 were actually in China, whereas China's inclusion as
469 a country for U.S. and EU-headquartered companies in phase
470 one trials were only about five percent each. Some U.S.-
471 headquartered sponsors are indeed initiating early trials in
472 China, but even more so in Australia to confirm the asset's
473 viability before coming to the U.S. due to cost and speed to
474 signaling advantages.

475 Second, it is not the FDA review timelines, but rather

476 the IND package preparation that drives ex-U.S. first-in-
477 human starts. Consistently our sponsors, particularly
478 emerging biopharma companies who drive over 75 percent of the
479 global phase one starts, cite time and resources to assemble
480 the IND package itself as the barrier to initiate trials in
481 the U.S. Many start their first trial ex-U.S. while
482 concurrently building their full IND package here in the
483 U.S., and we appreciate that FDA and HHS are taking
484 meaningful steps to address this through the Operation
485 Trialblazer.

486 Congress should consider encouraging the definition and
487 use of fit for first-in-human minimum data sets based on
488 complexity and risks to support the safe-to-proceed
489 decisions. And that would, in essence, speed the U.S. starts
490 without lowering the standards or compromising patient
491 safety.

492 Third, this fit for first-in-human focus at the IND
493 application review will defer important conversations later
494 in development. However, this will require more flexibility
495 and capacity for FDA reviewers to provide that scientific
496 advice as assets advance. Emerging biopharma companies and
497 their investors place an extremely high value on FDA feedback
498 to inform their development decisions and to attract further,
499 often staged investments beyond first-in-human. Without the
500 support, we may certainly gain more first-in-human trials

501 with speed to signal, but see costly rework and delays in
502 later stages.

503 Congress should consider supporting the FDA efforts to
504 adapt rolling review practices and increase capacity for
505 scientific advice, perhaps offering additional interactions
506 for early-stage companies who commit to start their trials
507 firstly in the U.S., a modernized IND review framework with
508 tailored first-in-human expectations and an FDA empowered to
509 provide more iterative scientific engagement to help our
510 sponsors start more early-phase trials here in the U.S. while
511 maintaining the patient protections and data integrity that
512 make the FDA the global gold standard.

513 And of course, IQVIA is continuing to be committed to
514 help the Congress, FDA, HHS, and, of course, other
515 stakeholders to enhance this clinical research efficiency
516 and, most importantly, helping our patients receive medicines
517 faster. Thank you.

518 [The prepared statement of Dr. Verst follows:]

519

520 *****COMMITTEE INSERT*****

521

522 *Mr. Griffith. The gentlelady yields back and I
523 appreciate that. I now recognize Ms. Winckler for her five-
524 minute opening statement.

525

526 STATEMENT OF SUSAN C. WINCKLER

527

528 *Ms. Winckler. Chairman Griffith, Ranking Member
529 DeGette, and members of the subcommittee, thank you for the
530 opportunity to testify today. I am Susan Winckler, chief
531 executive officer of the Reagan-Udall Foundation for the FDA.
532 I am a pharmacist and an attorney by training, and have
533 observed drug development from many vantage points including
534 as FDA's chief of staff under both Republican and Democratic
535 commissioners. Part of my role there involved negotiating
536 product safety agreements with China.

537 The Foundation is a non-profit created by Congress in
538 2007 to advance regulatory science and support FDA's mission,
539 so thank you for creating the organization. The insights I
540 offer today result from some of our recent convening
541 activity. Each produced dozens of specific stakeholder-
542 vetted recommendations, many of which offer insight into how
543 the United States can make its own drug discovery and
544 development system faster, clearer, and more capable. We
545 brought together clinical trial sites, research
546 organizations, FDA-regulated industry, patient groups, and

547 regulators to deliberate shared challenges with candor.

548 The resulting recommendations align with much of
549 Operation Trialblazer. This is encouraging. When the people
550 who run trials, treat patients, and generate evidence arrive
551 on their own as the -- at the same conclusions as the
552 regulatory agency, it is a strong signal that the reforms are
553 sound and ready to implement. But more can be done. The
554 goal is a stronger American system, and the surest way to
555 lead is to be the world's best place to bring therapies from
556 discovery to patients.

557 The single most cited reform across our convenings was
558 the need for clear, phase-appropriate FDA requirements and
559 more opportunities to interact with and learn from the FDA.
560 Specifically, adapting investigational new drug submission
561 requirements according to the risk presented, as Dr. Verst
562 mentioned, and triaging the review can accelerate routine
563 INDs and focus limited FDA resources to higher-risk INDS to
564 more novel interventions and emerging science.

565 There is also benefit in informal interactions between
566 sponsor and regulator, shared learning involving multiple
567 parties, and simply using clearer language. For example,
568 using the phrase "clinical hold" when FDA pauses an IND
569 application before any human has received a dose can lead
570 people to think that clinical work has stopped. It is a
571 simple "notice of additional information required" would be

572 phrasing that is clearer and less alarming both to patients
573 and investors.

574 FDA can clarify guidance and streamline review, and it
575 should. But innovation is not advanced simply by refining
576 regulations. FDA cannot build physical and human
577 infrastructure, and some needed reforms sit with other HHS
578 divisions such as CMS. Congress, including this
579 subcommittee, has a considerable role to play. I share three
580 areas for subcommittee exploration.

581 First, we lack a clear national picture of America's
582 phase one trial capacity. Which academic medical centers and
583 units can safely activate first-in-human trials, and how much
584 of that capacity is at risk? How does that landscape compare
585 with the independent, for-profit sites that may focus more on
586 healthy volunteer studies? The stakeholders we gathered
587 recommend building a dedicated network of phase one capable
588 sites, including centers of excellence that apply policies
589 and practices intended to speed time to trial. Such work
590 requires Federal resources and evaluation of the center of
591 excellence concept.

592 Second, where trial sites are located is uneven across
593 the country. The number of trial sites is also declining
594 under financial pressure. In addition to expanding
595 opportunities like the hub-and-spoke model for clinical
596 trials, it is also an opportunity to reimagine the screening

mechanisms for clinical trial participation. Reimagine them to be patient-centric rather than trial-centric. Further, harmonizing legal structures regarding permissible incentives and support for clinical trial participants, which can be essential for offsetting the burden in research participation, would serve patients and innovation alike.

Third, current contracting and budget negotiations between trial sponsors and research sites simply take too long and add unneeded complexity to trial management. Congress could encourage sponsors and trial sites to generate and use common processes and multi-sponsor, multi-site templates to help shorten the time from IND approval to patient dosing.

In closing, we need to create a regulatory system that keeps pace with scientific advances. HHS has chartered a sound course, and what remains is squarely Congress's opportunity, including assessing and strengthening our national phase one capability, removing barriers, and, where needed, amending the Food, Drug, and Cosmetic Act. Our Foundation will continue to support this ecosystem. We stand ready to help.

[The prepared statement of Ms. Winckler follows:]

*****COMMITTEE INSERT*****

622 *Mr. Griffith. Thank you very much. I now recognize
623 Dr. Kowalski for his five-minute opening statement.

624

625 STATEMENT OF AARON J. KOWALSKI

626

627 *Dr. Kowalski. Good morning, Chairman Guthrie, Ranking
628 Member Pallone, Chairman Griffith, and Ranking Member
629 DeGette, and members of the subcommittee. And thank you,
630 Ranking Member DeGette, for your kind introduction and for
631 all you have done for your daughter and our community. Thank
632 you for the opportunity to testify before you today on a
633 subject that is critical not only for our nation's
634 competitiveness, but for every American who struggles with
635 chronic and serious illness.

636 I am Dr. Aaron Kowalski, chief executive officer of
637 Breakthrough T1D, formerly JDRF, the leading global research
638 and advocacy not-for-profit organization for type 1 diabetes
639 or, as we say, T1D. Our purpose is to make everyday life
640 with type 1 diabetes better while driving towards cures. We
641 do this by investing in promising research, educating the
642 public, and working with the government, like this esteemed
643 committee, to address critical issues in the T1D community.

644 I am a scientist by training, but I also live with type
645 1 diabetes, as does my brother, Steve. So I know firsthand
646 the constant challenges of managing this disease, as well as

647 the promising science on the horizon. I have also worked
648 with FDA for nearly 20 years, and I can say with pride that
649 my family and our broad type 1 diabetes community has
650 benefitted directly from American innovation and FDA
651 standards. There is work to do, but I want to commend the
652 FDA's new leadership. In just a short time they have shown a
653 real commitment to becoming a forward-leaning partner in
654 innovation that the T1D community needs.

655 Type 1 diabetes is an autoimmune disease that can affect
656 anyone at any age. Once you have type 1 diabetes, you rely
657 upon insulin for the rest of your life. Without insulin,
658 type 1 diabetes is fatal. Insulin, though, is not a cure for
659 type 1 diabetes. The disease is unrelenting to manage, even
660 with the amazing progress that we have made over the last
661 number of years. It is 24/7, 365, and it can have serious
662 complications and consequences. High blood sugar can lead to
663 diabetic complications such as heart disease, strokes,
664 amputations, and blindness. But on the flip side, too much
665 insulin at the wrong time can lead to hypoglycemia or low
666 blood sugar, which can be life-threatening in just minutes.

667 So Breakthrough T1D has invested over \$2.5 billion in
668 type 1 diabetes research to try to solve these problems. But
669 we can't cure type 1 diabetes alone. We are so grateful for
670 the bipartisan leadership of Representatives DeGette and
671 Bilirakis and the strong support of this committee for

672 continuing support of T1D research and the Special Diabetes
673 Program.

674 America has a long and proud history of leading T1D
675 research, innovation, and treatments. But the rest of the
676 world is catching up. I saw this firsthand during recent
677 visits to China in December, and Australia in April. That
678 said, our global leadership in T1D innovation is ours to
679 lose. America is well equipped to continue to lead. NIH
680 research funding and FDA regulatory approvals are the
681 linchpins to that success. It would be a tragedy to fall
682 behind because we failed to keep pace with the speed of
683 innovation. But that has begun to happen.

684 Transplantation of deceased donor islet cells is an
685 example of this. These transplants have been safely
686 performed abroad for decades, benefitting select patients who
687 qualify. But these same deceased donor islets here in the
688 United States remain largely inaccessible for research and
689 transplantation because FDA regulates them as drugs instead
690 of organs.

691 Clinical trials are another challenge. C-peptide, for
692 example, is the best, simplest measure of your body's insulin
693 production. Yet in clinical trials FDA hasn't granted full
694 approval for using C-peptide as an endpoint. Antiquated
695 requirements needlessly prolong and complicate clinical
696 trials and can deter innovation and investment. I commend

697 the recently-announced trialblazer pilot aimed at modernizing
698 clinical trials. Breakthrough T1D has also stepped in to
699 help by publishing road maps for T1D cell therapies, giving
700 more clarity to navigate FDA pathways.

701 A robust FDA, one that is stable and well resourced,
702 consistent and transparent, modern in its clinical trial and
703 endpoint policies, and that incorporates patient perspectives
704 is essential to our global leadership in biopharmaceutical
705 innovation and manufacturing. An FDA with these qualities
706 will be ready for the next wave of T1D innovation, which is
707 already right here at our doorstep. Cell therapies are
708 already resulting in insulin independence. One pivotal trial
709 is happening in the U.S. right now. With the right approach
710 we can be the first country to approve and manufacture
711 scalable cures for T1D.

712 My message is simple: We have the capacity to end type
713 1 diabetes and many other chronic and debilitating illnesses
714 if FDA adapts and modernizes to keep pace with scientific
715 progress.

716 Thank you again for inviting me to testify, and I look
717 forward to questions.

718 [The prepared statement of Dr. Kowalski follows:]

719

720 *****COMMITTEE INSERT*****

721

722 *Mr. Griffith. Thank you, and I now recognize Dr. Hwang
723 for his five-minute opening statement.

724

725 STATEMENT OF THOMAS HWANG

726

727 *Dr. Hwang. Chairman Griffith, Ranking Member DeGette,
728 Chairman Guthrie, Ranking Member Pallone, and members of the
729 subcommittee, thank you very much for the opportunity to
730 testify. My name is Thomas Hwang. I am a physician and
731 health policy researcher at Harvard Medical School and the
732 Brigham and Women's Hospital in Boston, where I lead the
733 Cancer Innovation and Regulation Initiative. My research
734 focuses on the regulation, clinical development, and payment
735 of new medicines, medical devices, and other interventions in
736 cancer care. All views here are my own only.

737 The United States' leadership in biomedical innovation
738 is built on strong and stable investments in research.
739 Nearly every drug entering the U.S. market is linked to NIH-
740 supported science. Early stage clinical testing is at the
741 leading edge of this innovation. These studies have special
742 regulatory and scientific significance for three main
743 reasons.

744 First, early-stage studies of new therapies are often
745 located, although not always, where the scientific discovery
746 occurs.

747 Second, first-in-human studies have heightened safety
748 obligations because they traditionally test healthy
749 volunteers. In oncology, early-stage trials may enroll
750 affected patients and can serve as a pivotal study supporting
751 regulatory approval.

752 And third, unforeseen safety issues can and do occur
753 when pre-clinical models fail to predict actual human
754 response.

755 So where early-stage trials are conducted matters both
756 for the safety of our patients and for innovation leadership
757 more broadly. Our research reviewed the phase one studies
758 and other early-stage testing for novel cancer drugs approved
759 by the FDA. We found that the share of these studies with at
760 least one U.S. trial site declined substantially over the
761 past seven years. During this time early-stage trial
762 activity in China increased. As recently as 2018 cancer drug
763 approvals did not involve any first-in-human study site in
764 China. Since then, the share of early-stage cancer trials
765 with any Chinese trial site nearly tripled, and the share of
766 first-in-human studies -- again, in cancer -- with any
767 Chinese site doubled. Our research indicates that rising
768 Chinese contributions to early-stage clinical development now
769 extend to the first-in-human and pivotal early-stage studies
770 that underpin FDA approval.

771 Our patients benefit from access to the world's best

772 scientific discoveries that will ultimately generate safe,
773 effective, and affordable treatments for their conditions.
774 We should welcome competition in the global research
775 enterprise, but such competition has to be fair, transparent,
776 and ethically sound. Recently the FDA has raised questions
777 about data from single country and non-U.S. clinical trials
778 that may not be generalizable to patients here. For example,
779 these studies may use a suboptimal control group that does
780 not reflect standard of care in the U.S. The entire
781 biomedical enterprise suffers when clinical research moves to
782 sites with inferior standards for data integrity, safety, and
783 ethical protection of participants.

784 As this subcommittee looks forward to upcoming
785 reauthorization of user fee agreements, I want to share three
786 policy proposals that could support our nation's leadership.

787 First, improved transparency can accelerate early-stage
788 clinical testing and ensure efficient regulatory review.
789 This helps sponsors avoid similar mistakes and learn from
790 past development failures. The FDA has recently launched an
791 initiative to provide more information on why new drugs are
792 not approved, including publicly releasing complete response
793 letters. Congress should provide explicit statutory backing
794 for the FDA's transparency initiative and extend it to
795 include reasons for halted early-stage trials and a summary
796 of the steps that are taken to resolve these suspensions.

797 Second, Congress should explore ways to increase global
798 standards for clinical research that protects both patients
799 and U.S. competitiveness. The FDA remains the global model
800 for regulatory approval of new therapies, and the U.S. is the
801 most lucrative market for these products. So the agency's
802 priorities shape the trajectory of development programs
803 around the world. Congress could examine requiring that a
804 minimum percentage of non-U.S. trial sites undergo FDA
805 inspection.

806 Third, and finally, as part of the FDA's Operation
807 Trialblazer the FDA could pilot coordinated submission and
808 review of investigational new drug and investigational device
809 exemption applications with international partners which
810 allow new drugs and devices to begin clinical testing.

811 Ultimately, any durable effort to promote U.S. clinical
812 development requires investments in public support for
813 foundational research and adequate staffing at FDA.

814 Thank you for the invitation, and I welcome any
815 questions.

816 [The prepared statement of Dr. Hwang follows:]

817

818 *****COMMITTEE INSERT*****

819

820 *Mr. Griffith. Thank you, and I now recognize Mr.
821 Bollyky for his five-minute opening statement.

822

823 STATEMENT OF THOMAS K. BOLLYKY

824

825 *Mr. Bollyky. Chairman Griffith, Ranking Member
826 DeGette, distinguished members of the subcommittee, thank you
827 for this opportunity to testify today. I am Tom Bollyky, the
828 Bloomberg chair in global health at the Council on Foreign
829 Relations. I recently co-led a year-long analysis with
830 biopharmaceutical specialists, China scholars, industrial
831 policy experts to identify the true scope of U.S. dependence
832 on China in the biopharmaceutical sector and to recommend
833 policies to reduce it.

834 My remarks today build on that report, focusing on,
835 first, the consequences for patients, public health, and
836 biosecurity if the U.S. cedes its global leadership in drug
837 discovery and development to China; and second, how Congress
838 and FDA can streamline the early-stage clinical development
839 process without undermining patients' timely access to safe
840 and effective medicines.

841 Let's start with how drug discovery is shifting to China
842 and the potential consequences. China now offers pathways
843 for conducting first-in-human trials that are three to five
844 times faster and one-third to half the cost of the U.S.

investigational new drug process. Chinese trials are supported by a dense ecosystem of contract research, development, and manufacturing organizations, hospitals capable of enrolling hundreds of treatment-naïve patients at a single site, heavy state subsidies, and a permissive scientific ethics system. The ability to conduct first-in-human trials quickly and at low cost enables Chinese biotech firms to determine which compounds hit their biological targets to refine them, leapfrog more slowly-moving U.S. competitors, and to attract licensing -- vital licensing deals.

The results speak for themselves. China's share of global clinical trial starts has risen from 1 percent in 2009 to 30 percent in 2024, rivaling the U.S. share. By one estimate, one-third of the pharmaceutical industry's global licensing spending in 2025 involved assets originating in China. If this trend continues, it threatens to reallocate the early-stage biopharmaceutical pipeline away from U.S. start-ups and hollowing the American innovation ecosystem. The risk of that will be threefold.

First, there is a biosecurity risk if China can create strategic and economic leverage by choking off supplies of innovative medicines on which U.S. patients and hospitals rely. China has already weaponized rare earth critical minerals and agricultural products, targeted pharmaceutical

870 supply chains in Japan and India, and threatened to do so
871 more broadly.

872 Second, there is a risk to public health if the U.S.
873 capacity to do innovative drug development diminishes and we
874 cannot rapidly produce effective countermeasures in a crisis,
875 as occurred in COVID-19.

876 Third, there is a risk to patient safety because FDA
877 cannot effectively oversee the level of clinical trial
878 activity now occurring in China, which means U.S. patients
879 and health care providers may not be able to fully rely on
880 the resulting data. One recent study found that just 20 GCP
881 inspections, less than 1 percent of FDA's GCP inspections
882 between 2016 and 2023, occurred in China while 93 percent of
883 those inspections happened in the U.S.

884 So what should Congress and the FDA do?

885 First, we need to make our own system better by adopting
886 the operational features of other well-regulated nations that
887 have modernized early clinical trial oversight without
888 sacrificing patient safety or scientific integrity. Congress
889 and the FDA should authorize a narrow, expedited pathway for
890 well-characterized drug platforms and lower-risk first-in-
891 human studies such as Australia has pioneered. It should
892 combine that with a program that invests in dedicated
893 university and hospital phase one sites, as Norway has, that
894 use public-private partnerships to inform trial design,

895 central IRBs, and ensure diverse trial subject populations
896 that draw from rural areas too.

897 Second, FDA should reduce dependance on Chinese contract
898 biomanufacturers by making it easier for U.S. firms to adopt
899 advanced manufacturing technologies and AI-driven process
900 innovations, and by being better prepared to authorize
901 temporary importation of essential medicines should China
902 seek to cut off supplies to them.

903 Third, Congress needs to continue to invest in the
904 enabling environment that makes the U.S. biomedical industry
905 thrive, including our Federal agencies and especially FDA and
906 NIH. Rehiring is occurring, but FDA staffing is down from
907 2025. The agency has lost real experience and expertise.
908 Adding a user fee to enhance the number and quality of GCP
909 inspections for IND submissions with foreign clinical trial
910 data would help boost FDA's inspection capacity.

911 In conclusion, the same dynamic that hollowed out U.S.
912 generic drug manufacturing is now playing out in
913 biotechnology, but at an earlier stage when FDA and
914 congressional intervention would be effective and is thus
915 more urgent. The time to act is now. I thank the
916 subcommittee for the opportunity to testify and stand ready
917 to answer any questions.

918 [The prepared statement of Mr. Bollyky follows:]

919

920 *****COMMITTEE INSERT*****

921

922 *Mr. Griffith. I thank you and all the witnesses. We
923 will now begin questioning. I would ask that members not
924 begin a new question -- let me underline that -- not begin a
925 new question with about 10 or 15 seconds to go, because that
926 would hopefully move us along a little quicker. I would
927 encourage members to submit written questions for the record,
928 and I will tell everybody -- I have already leaned back and
929 told my team a couple of questions for the record because I
930 know I can't get to them all that I want to get to. But you
931 all have presented a lot of interesting ideas for us to
932 consider.

933 I now recognize myself for five minutes and I am going
934 to start with Dr. Verst.

935 Dr. Verst, the -- I appreciate you talking about the
936 IND. And the FDA has recently put out a proposed expedited
937 investigational new drug program to accelerate clinical
938 research and development. Do you think their plan will work
939 or do we need to do more?

940 *Dr. Verst. I do think that it will take the collective
941 ecosystem to be leaning in to ensure the success, but I think
942 it is a terrific beginning in recognizing the problems and
943 the solutioning that will be required in order to achieve
944 that.

945 *Mr. Griffith. All right. Now, one of the things you
946 mentioned that I have been toying with in my own mind -- and

947 you just touched on it tangentially, so maybe I heard what I
948 wanted to hear -- was you mentioned risk, and it has bothered
949 me that when you have low risk -- sometimes FDA wants
950 everything done, you know, according to their standard
951 process. But if you have low risk, should we be creating a
952 second standard that says, you know, it is not -- that we
953 don't know if it works for what it says it is going to work
954 for, but the risk is so low, if you want to try it you can,
955 and create a new standard? Should we do that? Because that
956 would get them to give us the data to whether or not it
957 works.

958 *Dr. Verst. Yes.

959 *Mr. Griffith. Your thoughts?

960 *Dr. Verst. I do believe that is a terrific question.
961 And I think it is really more risk based in our approaches.
962 And we have got many examples of that within the clinical
963 development arena supported by the FDA. For instance, risk-
964 based quality monitoring. And I think having the ability to
965 be more, if you will, weighted -- and of course, that is
966 adaptive with regard to our approaches. And being very
967 focused on that risk-based profile will enable us to speed
968 along processes and reviews, but not jeopardizing patient
969 safety nor data integrity along the way.

970 *Mr. Griffith. And that is our concern -- or my
971 concern, as well. But do they need a nudge from Congress? I

972 will get an answer from you later, and I will do a written
973 question on that one.

974 [The information follows:]

975

976 *****COMMITTEE INSERT*****

977

978 *Mr. Griffith. Ms. Winckler, you highlighted how trial
979 capacity is currently unevenly distributed across the
980 country, with many patients living a long way from the trial
981 site. Well, that is that -- welcome to my district.

982 *Ms. Winckler. Absolutely.

983 *Mr. Griffith. I am a long way from any place that does
984 that. I mean, people say, well, why don't you just go to
985 Charlottesville? Well, in parts of my district it is not
986 Charlottesville you would be looking at, you would be looking
987 at Cleveland because you are closer to Cleveland than you are
988 to Charlottesville, even though you are in the state of
989 Virginia.

990 So what can we do to get more sites involved in the
991 clinical trials?

992 And I specifically want to look at community health
993 centers, particularly federally-qualified ones.

994 *Ms. Winckler. Yes.

995 *Mr. Griffith. What say you?

996 *Ms. Winckler. Yes, one of the ways to enhance access,
997 and so to get into areas where they aren't today, is to make
998 better use of and clarify the rules for what is called a hub
999 and spoke model. Right? So that you have the really
1000 important but paperwork-heavy responsibilities of the hub --
1001 thinking about this is how we run trial sites -- and they
1002 hold some of that responsibility for the spoke so that in

1003 communities you could have clinical trials available through
1004 the clinicians in the community, and they will operate
1005 according to the rules and regulations, you know, and the
1006 oversight of the hub, but it helps structure the mechanisms
1007 so that you aren't trying to recreate in a local area where
1008 you might have just a couple of physicians who could do some
1009 things. Let's empower them to do those some things in
1010 collaboration with a hub.

1011 *Mr. Griffith. And I would be correct that telemedicine
1012 will help with that, yes?

1013 *Ms. Winckler. Yes, there are definitely some
1014 opportunities in remote monitoring and telehealth.

1015 *Mr. Griffith. Thanks.

1016 Dr. Hwang, your thoughts on how we can get more sites
1017 like community health centers involved in these early-stage
1018 clinical trials?

1019 *Dr. Hwang. Thank you for the question.

1020 Well, we know from the Australian experience and from
1021 other countries that public investment in our hospitals and
1022 health systems is critical to improving the capacity that
1023 they have in running clinical trials, and having a
1024 centralized network of these institutions that could be
1025 centers of excellence could speak to kind of your concerns
1026 today.

1027 *Mr. Griffith. All right. If you can do it in 30

1028 seconds, Ms. Winckler, how can using real-world evidence or
1029 natural history studies in trial designs help new treatments
1030 for rare diseases?

1031 *Ms. Winckler. It could be very helpful in helping to
1032 structure the trials, as well as to -- after perhaps a
1033 product is available, to do follow-up and make sure that we
1034 have the performance that was predicted in the initial piece.
1035 So that data both before submission and approval and after
1036 approval, we need to make better use of all of that
1037 information.

1038 *Mr. Griffith. I appreciate it.

1039 *Ms. Winckler. It is rare data.

1040 *Mr. Griffith. You did great. I now yield back and
1041 recognize Ms. DeGette, ranking member of the subcommittee,
1042 for her five minutes.

1043 *Ms. DeGette. Thank you so much, Mr. Chairman, and
1044 thanks to everybody for their testimony. Really, it is very
1045 rare that we see people from all aspects of the industry sort
1046 of rowing together in the same direction. And I think we
1047 know what we need to do.

1048 The Federal Government, and the FDA in particular,
1049 really do have an immense impact on biomedical research and
1050 with grants and other collaborations. But as all of you
1051 know, our resources are not unlimited, so we have to fund
1052 high-quality, basic research and impactful public health

1053 projects. Dr. Kowalski, I wonder if you can talk to us about
1054 the importance of merit-based peer review process.

1055 *Dr. Kowalski. Well, merit-based peer review is

1056 fundamental to making good decisions that are unbiased, that
1057 have input from expert scientists, and are aligned with the
1058 unmet needs in science to drive towards clinical benefits.
1059 So it is fundamental to everything we do in science.

1060 *Ms. DeGette. So OMB recently proposed a rule that
1061 dramatically changes how the Federal Government would
1062 evaluate Federal funding and how grantees can use the Federal
1063 dollars. What would the impact of that rule on the peer
1064 review process be?

1065 *Dr. Kowalski. I think any scientist would tell you
1066 that we need to keep politics out of science. It is critical
1067 that the science is evaluated on the merits, on the evidence,
1068 and that we drive forward without the influence of politics.

1069 *Ms. DeGette. And so you think that the proposed rule
1070 would really adversely impact the peer review process?

1071 *Dr. Kowalski. I think so, and we have signed on with a
1072 coalition to lodge our feelings about this issue.

1073 *Ms. DeGette. Okay. Now, international research
1074 collaborations have made important contributions to the
1075 science of type 1 diabetes. Isn't that correct?

1076 *Dr. Kowalski. Absolutely.

1077 *Ms. DeGette. And how would that proposed rule treat

1078 those international collaborations?

1079 *Dr. Kowalski. Well, we have seen already in a number
1080 of groups that we fund barriers be put up to some of these
1081 collaborations. For example, I just came from a meeting
1082 where we funded an international study on pregnancy and
1083 diabetes. Those investigators often rely on agreements
1084 through NIH multi-center study grants, and those barriers we
1085 absolutely do not want to see happen.

1086 *Ms. DeGette. Okay. Can you just briefly tell us about
1087 the TEDDY study?

1088 *Dr. Kowalski. So this is an amazing study. It is one
1089 of the largest databases currently at NIH that is aiming to
1090 identify the causation of type 1 diabetes. Type 1 diabetes
1091 has a genetic component, but even an identical twin who gets
1092 type 1, it is only about a 50 percent risk the other one will
1093 get it, which says there is something in the environment that
1094 is causing type 1.

1095 When we look at the use of AI in the future, I really
1096 believe the combination of genetics and this environmental
1097 data from TEDDY will really help us understand what is
1098 causing type 1 diabetes --

1099 *Ms. DeGette. So one of the provisions of the proposed
1100 rule would let political appointees terminate grant awards,
1101 so that would effectively kill projects at any time for any
1102 reason. What would happen if the TEDDY -- this huge TEDDY

1103 collaboration that you are talking about were cut off halfway
1104 through because maybe a political appointee didn't like the
1105 word "environmental" ' '?

1106 *Dr. Kowalski. It would be a travesty.

1107 *Ms. DeGette. It would totally destroy the results of
1108 that study.

1109 *Dr. Kowalski. We -- I mean, we haven't -- we don't --
1110 we have certainly benefitted from TEDDY with a number of
1111 seminal learnings, but the ultimate goal of understanding the
1112 genesis of type 1 diabetes is not there yet.

1113 *Ms. DeGette. Right.

1114 *Dr. Kowalski. And that is one of the most important
1115 studies that --

1116 *Ms. DeGette. Right. Dr. Hwang, I want to ask you
1117 quickly, what kind of work does FDA fund externally?

1118 *Dr. Hwang. The FDA has a lot of important grant
1119 mechanisms, specifically in the Office of Orphan Products.
1120 FDA grants to rare disease clinical trials, including those
1121 for ALS, have supported new product approvals. And all of
1122 that research is at risk.

1123 *Ms. DeGette. And in your view, if this rule I have
1124 been talking about were implemented, would that undercut that
1125 external work?

1126 *Dr. Hwang. I would have concerns that it would.

1127 *Ms. DeGette. Dr. Kowalski and Hwang, do you think --

1128 and I am just going to ask you -- do you believe the proposed
1129 rule should be withdrawn?

1130 I will start with you, Dr. Hwang.

1131 *Dr. Hwang. I am still reviewing the proposed rule, but
1132 as written I have serious concerns before it is implemented.

1133 *Ms. DeGette. And Dr. Kowalski?

1134 *Dr. Kowalski. I think we share that -- those concerns.

1135 *Ms. DeGette. Okay, thanks.

1136 Thank you so much, Mr. Chairman. I yield back.

1137 *Mr. Griffith. The gentlelady yields back. I now
1138 recognize the chairman of the full committee, Mr. Guthrie of
1139 Kentucky, for his five minutes of questions.

1140 *The Chair. Thank you very much. Thank you.

1141 So Ms. Winckler, the Reagan-Udall Foundation has
1142 extensive experience convening stakeholders to identify
1143 opportunities to accelerate drug development, particularly in
1144 areas where there are few treatments such as psychiatric
1145 substance use disorders, particularly psychedelics we are
1146 referring to.

1147 As the Administration takes action on accelerating
1148 development and access to therapies for these types of
1149 conditions, where do you see the greatest opportunity for FDA
1150 and external stakeholders to work together to bring safe and
1151 effective treatments?

1152 *Ms. Winckler. So there is a few opportunities, but in

1153 particular assuring that the endpoints that we are pursuing
1154 -- so what the treatments intend to effect -- match what
1155 patients are looking for. So that is one of the components.

1156 And then there is also the important conversation
1157 between the product developers and the regulator for what is
1158 -- what should be measured and how do those clinical trials
1159 need to be conducted. There certainly have been a number of
1160 those conversations that have taken place. And now, as that
1161 work continues, it will be important to share the learnings
1162 as some products move through that process what can other
1163 developers --

1164 *The Chair. Because one of the issues, like with
1165 psychedelics, you can't really do a placebo trial because,
1166 obviously, if you have taken a placebo psychedelic, you know
1167 you are taking the placebo psychedelic.

1168 *Ms. Winckler. Right, that is one of the challenges.
1169 So FDA has a guidance on that to try and help parse out, you
1170 know, how the trial should be done so that --

1171 *The Chair. They just did that in the last couple of
1172 days. You think that is going to be effective, their
1173 guidance? Do you like the guidance they put out in the last
1174 couple of days on that?

1175 *Ms. Winckler. So we have had the privilege to convene
1176 some of the conversations that contributed to that, so we
1177 don't take a position on the guidance. But we -- I am aware

1178 that there were constructive conversations that helped make
1179 sure that it was reflecting the needs of the community.

1180 *The Chair. Because we have a number of colleagues in
1181 Congress who were combat veterans and have had this treatment
1182 and have lots of friends, and they have talked of this
1183 treatment and feel it is really effective. And we need to
1184 figure -- hopefully, we can figure out a pathway, if it is
1185 effective, to find it is effective and give access to it. So
1186 thanks.

1187 So Dr. Kowalski, in Breakthrough T1D you have worked
1188 closely with the FDA and product developers to advance all
1189 the innovative clinical designs and trials with endpoint
1190 development for therapies and ultimately to benefit patients
1191 with type 1 diabetes. Can you describe how early
1192 collaboration between the FDA sponsors and patient
1193 organization contributed to that success, and what lessons
1194 can be applied more broadly to accelerate development?

1195 *Dr. Kowalski. Yes, thank you, Mr. Chairman. This is
1196 such an important piece, I think, of the progress we have
1197 seen in type 1 diabetes has been a collaboration between
1198 regulators and patient advocacy groups like ours.

1199 You know, there is a hesitancy often in medicine to take
1200 risk. But I think one of the under-appreciated things is
1201 living with type 1 diabetes is risky, and we certainly saw
1202 significant progress made back when I worked quite a bit on

1203 diabetes devices, for example, with collaboration,
1204 understanding risk benefit, safety, and efficacy, and driving
1205 together to doing the right trials and having the right
1206 pathways to come through FDA.

1207 *The Chair. Okay, thank you.

1208 And so Dr. Verst, we have heard increasing concerns that
1209 other countries are reducing the time it takes to activate
1210 clinical trials and enroll patients, making them more
1211 attractive locations for early-stage development. What are
1212 the potential risks if we as Americans fail to remain
1213 competitive with clinical trials?

1214 And what -- particularly, what -- so the risk and what
1215 we can do to have the greatest impact on ensuring America
1216 remains the preferred location.

1217 *Dr. Verst. Mr. Chairman, that is a terrific question,
1218 and I think the largest risk is not getting on the other side
1219 of approvability, better medications to our patients faster,
1220 the largest risk. I think what we can do is actually putting
1221 more innovation at the top of the funnel, and that is what is
1222 truly at risk here from a China perspective, and that is more
1223 translational, if you will, medicine in science with more
1224 assets, drug discovery, occurring, you know, early in the
1225 process.

1226 And then, as I mentioned in my remarks, simplifying and,
1227 importantly, accelerating and doing more of that adaptive

1228 approach, more of the risk approach to actually hasten the
1229 process.

1230 And then finally, clarification. Especially with
1231 biotech pharma companies, they need that early engagement
1232 that you alluded to earlier much more preemptively, even in
1233 the pre-IND phase. And being more, if you will, predictive
1234 and reliable, especially because of the backing of investors.
1235 They really encourage that FDA feedback and insights.

1236 *The Chair. We need to have the risk approach because,
1237 I mean, if you are just trying to cure the sniffles, you want
1238 to make -- you want a lot of tests to make sure there is no
1239 side effects. But that is what the President is for -- in
1240 the first term and now pushed right to try. Some people are
1241 in a situation where this is their only hope, and this is
1242 something that is going to cost them their lives.

1243 And so we -- and then with the -- our suicide, that is
1244 what some of my colleagues talk about with some of the
1245 treatments that I mentioned earlier. So we are focused on it
1246 and we look forward to working with you.

1247 And I yield back.

1248 *Mrs. Harshbarger. [Presiding.] Okay, the gentleman

1249 yields back, and I now recognize the ranking member of the
1250 full committee, Mr. Pallone, for his five minutes of
1251 questions.

1252 *Mr. Pallone. Thank you, Chairwoman.

1253 I mentioned in my opening statement we need to ensure
1254 there is a robust research infrastructure in the U.S.,
1255 something that has been seriously threatened by the Trump
1256 Administration. In May the Trump Administration took another
1257 harmful step to decimate our public research when OMB
1258 overhauled the Federal grant rules and gave political
1259 leadership final authority over discretionary award
1260 decisions. This politicized the science and threatens the
1261 independence of scientific research in the United States.

1262 On Monday a group of 57 organizations representing
1263 patients, caregivers, and families that rely on drug
1264 discovery wrote to congressional leadership warning of, I
1265 quote, "profound implications on biomedical research and the
1266 future of treatments and cures in the United States.'`

1267 And I would like to ask unanimous consent, Madam Chair,
1268 to introduce this into the record, if you will.

1269 *Mrs. Harshbarger. Without objection, so ordered.

1270 [The information follows:]

1271

1272 *****COMMITTEE INSERT*****

1273

1274 *Mr. Pallone. Thank you. So every Democrat on this
1275 subcommittee wrote to the OMB director earlier this week to
1276 rescind this politicizing rule.

1277 I wanted to ask Dr. Hwang, your testimony speaks to the
1278 vital role of U.S. public investment in biomedical
1279 innovation. Why is it important to have an ecosystem that is
1280 supportive of this investment, including on foundational
1281 research?

1282 Your testimony notes that the U.S. has historically been
1283 the engine for biomedical innovation. However, your research
1284 now shows the growth in Beijing, China's action in this
1285 space. So basically, let me ask you first about the vital
1286 role and then I have a second question, Doctor.

1287 *Dr. Hwang. So multiple studies have shown that
1288 virtually every new drug that enters the U.S. market is
1289 supported by NIH science. Nearly a third of all Federal
1290 patents are based on Federal research. And we can't hope to
1291 increase early-stage clinical development without sustainable
1292 investments in our science.

1293 *Mr. Pallone. Well, thank you. But what evidence have
1294 you seen regarding the rising Beijing, Chinese contributions
1295 to early-stage clinical development?

1296 *Dr. Hwang. So what our study has shown in
1297 collaboration and in knowledge of other studies that are
1298 going on as well is that it is not declining U.S.

1299 productivity in science, but rather increasing Chinese
1300 investments in early-stage research and in the trial networks
1301 that we are lacking here. And what our studies showed is
1302 that in the important studies that underlie U.S. cancer drug
1303 approvals, now a significant portion of them are being done
1304 in China and being transported here.

1305 *Mr. Pallone. It is also part of this brain drain --
1306 you know, we keep reading about scientists that would
1307 normally, you know, either come to the United States or even
1308 American scientists that are leaving the country to go
1309 elsewhere -- China, Western Europe, elsewhere -- how is that
1310 contributing to the drug development or lack thereof, or
1311 slowing here in the U.S.?

1312 *Dr. Hwang. I think Congress has an important role in
1313 making the U.S. continue to be a leading destination for the
1314 world's best scientists, and I call upon this committee to
1315 help that.

1316 *Mr. Pallone. All right, thank you. I am also
1317 concerned that we are undermining the impact of -- or under-
1318 estimating, I should say, the impact of our dependance on
1319 Beijing. So let me ask Mr. Bollyky.

1320 In the report you published that you note that we need
1321 "a combination of measures to improve U.S. innovative
1322 capacity in the biotech space, combined with sensible
1323 security restrictions on Chinese firms.'" So what does your

1324 research show as a reason for clinical trial activity for
1325 promising interventions shifting away from the United States
1326 to Beijing, China?

1327 *Mr. Bollyky. Thank you for the excellent question.

1328 So part of it is a decline in U.S. competitiveness, not
1329 just vis a vis China for early-stage clinical trials, but
1330 also Australia and other markets as well. It is a
1331 cumbersome, burdensome process under the IND approach, and
1332 that has helped encourage activity to go abroad.

1333 But make no mistake, China's rise in this area is not
1334 just simply a matter of market forces. It is decades of
1335 coordinated state intervention and investment. And while it
1336 is critical as we -- the -- many of the witnesses have
1337 discussed here to invest in making the U.S. more competitive,
1338 we need to acknowledge that FDA acceptance of foreign
1339 clinical trial data depends on its compliance with good
1340 clinical practices that it has conducted under independent
1341 ethics review and the ability of FDA to inspect. And we
1342 cannot maintain that standard for the amount of activity in
1343 China. We have to have increased scrutiny over those trials
1344 and increased resources for FDA to do that.

1345 *Mr. Pallone. Thank you. Thank you very much.

1346 Thank you, Madam Chair.

1347 *Mrs. Harshbarger. Thank you. The gentleman yields
1348 back and I recognize myself for five minutes.

1349 You know, we have got two pharmacists on the panel and,
1350 as a pharmacist, I have spent my career helping patients, you
1351 know, benefit from innovative medicines and -- but innovation
1352 only matters if patients can safely access it. So I will
1353 start with you, Dr. Verst. Your testimony notes that Chinese
1354 companies overwhelmingly conduct their phase one work inside
1355 China. Should Congress view this primarily as a regulatory
1356 challenge or as a industrial competitiveness challenge?

1357 *Dr. Verst. I think it could be both in terms of the
1358 regulatory challenges and ensuring, for instance, at the end
1359 of the day -- we talked about representativeness.

1360 *Mrs. Harshbarger. Yes.

1361 *Dr. Verst. So increasingly we are seeing more global
1362 trials being executed outside of China by Chinese companies.
1363 And accordingly, you know, we pay very close attention,
1364 provisioning services to help them understand the regulatory
1365 implications at the FDA level. For instance,
1366 representativeness, which is being so critical in the data
1367 application.

1368 *Mrs. Harshbarger. Yes.

1369 *Dr. Verst. You know, and I also think, you know, from
1370 just a competitiveness perspective, that this phase one is --
1371 quite candidly, close on the heels is phase two. Right now
1372 the comparison of phase two trials in China as compared to
1373 the U.S. is increasingly becoming comparable. So I think --

1374 *Mrs. Harshbarger. Yes.

1375 *Dr. Verst. -- both threats.

1376 *Mrs. Harshbarger. Okay. Ms. Winckler, stakeholders

1377 often tell us that one FDA review division may expect
1378 something entirely different from another. So how important
1379 is it to get greater consistency across the FDA review
1380 divisions for encouraging companies to keep early-stage
1381 development here in the United States?

1382 *Ms. Winckler. So not only is the consistency
1383 important, but when -- sometimes there is a rationale for it
1384 in that the review division is looking at a different organ
1385 system, a different impact. And so what is important there
1386 is for the agency to share the scientific rationale for the
1387 differences not only with that sponsor, but could that
1388 information be available more broadly so that the
1389 inconsistencies are explained and provided in context.

1390 *Mrs. Harshbarger. I got you.

1391 Dr. Hwang, you found that China's role in early-stage
1392 oncology trials has grown substantially. I am a compounding
1393 pharmacist. So when we can't -- I have said this for 40
1394 years. There is a problem with APIs when you have them do
1395 what they are doing with everything, drop the price, corner
1396 the market, and then we are the ones that pay the price when
1397 you can't get those drugs. Should Congress be more concerned
1398 about losing scientific leadership or about relying on

1399 foreign-generated clinical data that FDA may have limited
1400 ability to inspect?

1401 *Dr. Hwang. Thank you. I think both of those should be
1402 the focus of this Congress.

1403 And specifically on the latter, as we heard from other
1404 members of this panel, the FDA does not inspect a significant
1405 number of trial sites in China. And what we do know from the
1406 literature is that many of these sites have inferior
1407 standards --

1408 *Mrs. Harshbarger. Yes.

1409 *Dr. Hwang. -- for data integrity and for safety. And
1410 so we should empower the FDA and substantially appropriate
1411 funds to do so, to do those inspections.

1412 *Mrs. Harshbarger. Yes. You know, I have talked to
1413 drug companies recently and they have been to China. They
1414 say their industrial facilities are very top of the line and
1415 they have an equivalent like the FDA. But I am like, how do
1416 we know that if we don't go inspect those? And it is --
1417 there is lots of questions there.

1418 Mr. Bollyky, you argued that dependance on China for
1419 drug development creates both economic and national security
1420 risks, and I agree. Can you explain why manufacturing
1421 domestic, early-stage clinical research capacity is now
1422 becoming a biosecurity issue and not merely an economic
1423 issue?

1424 *Mr. Bollyky. Thank you for the excellent question.

1425 So we have seen in other spaces trying to exercise a
1426 willingness to leverage dependance --

1427 *Mrs. Harshbarger. Yes.

1428 *Mr. Bollyky. -- on its supply chains and its system to
1429 gain economic or strategic advantage over other nations.

1430 This is not a fanciful possibility in the pharmaceutical
1431 space. They have done this with Japan already in terms of
1432 restricting access to dual use compounds that support
1433 pharmaceutical manufacturing. They have undermined India's
1434 attempt to develop supply chain resilience in the active --

1435 *Mrs. Harshbarger. Yes.

1436 *Mr. Bollyky. -- pharmaceutical ingredient and key
1437 starting materials space. They easily could do so in this
1438 context, so it puts us at real risk.

1439 *Mrs. Harshbarger. Yes, absolutely. I agree with
1440 everything. One point that stood out in your testimony, sir,
1441 was the limited number of FDA inspections conducted in China.
1442 And I have 11 seconds left, but if FDA cannot routinely
1443 inspect foreign trial sites how confident should Americans be
1444 in relying heavily on that data? It is just -- I don't know,
1445 this is a problem, and I came to Congress to fix this, and we
1446 are going to work on this. A lot of the suggestions you all
1447 have had we will take back to the FDA. So thank you for
1448 being here.

1449 I yield back and now I recognize my friend from
1450 California, Dr. Ruiz, for his five minutes.

1451 *Mr. Ruiz. Thank you. Thank you so much.

1452 We must, first and foremost, ensure the effectiveness
1453 and the safety of new innovations during the clinical trial
1454 process. That is that is ground zero. And as an emergency
1455 physician, I am concerned about the potential effects that
1456 the lack of participation in clinical trials may have on the
1457 effectiveness of new medical treatments and therapies for

1458 populations that are under-represented in these clinical
1459 trials, okay?

1460 So many barriers prevent patients from participating in
1461 clinical trials, especially individuals living in rural and
1462 underserved areas, working-class poor families. There are
1463 considerable financial considerations like can someone afford
1464 taking time off of work to participate in the trial, can they
1465 afford the transportation to the trial site and co-pays for
1466 the follow-up in the clinical trial visits and afterwards to
1467 the doctor? And there are also basic geographic
1468 considerations. If you are living in a rural community like
1469 many of my constituents, you might not be able to drive two
1470 hours to the nearest academic medical center to participate
1471 in their clinical trials, or you and your doctor might not
1472 even know about the trials and the opportunities that many
1473 patients may welcome, especially for end-stage illnesses in

1474 the first place.

1475 So these are barriers that we can and should address.

1476 That is why I introduced the bipartisan Clinical Trial
1477 Modernization Act, H.R. 3521, with Representative Pfluger
1478 from Texas. Imagine that, a Democrat from California working
1479 with a Republican from Texas doing the right thing. Isn't
1480 that how this should work?

1481 *Mrs. Harshbarger. Yes.

1482 *Mr. Ruiz. Right. So this bill would improve
1483 participation in clinical trials by addressing economic
1484 barriers and helping bring trials to underserved areas where
1485 the patients are.

1486 For the reasons I mentioned earlier, participant
1487 enrollment retention remain one of the biggest challenges in
1488 conducting clinical trials. Community health centers are
1489 trusted providers to 52 million patients in underserved
1490 communities, rural areas across the nation. Given their
1491 broad reach, CHCs can bring research closer to where people
1492 live and receive care and greatly expand delivery of clinical
1493 research to the underserved population.

1494 Ms. Winckler, in your written testimony you talk about a
1495 patient-centered approach. So what role could community-
1496 based providers play in improving patient recruitment and
1497 accelerating research?

1498 And how would bringing clinical trials into the

1499 community care settings change the way patients access
1500 innovative therapies?

1501 *Ms. Winckler. So one of the challenges in clinical
1502 trial recruitment is that patients may not be asked, and they
1503 are not asked because their local community provider does not
1504 have the information about what trial might be appropriate,
1505 might be helpful for them. So reimagining and having a
1506 patient-centric system would, in certain disease areas, you
1507 know, deploy against a community-based network, providing
1508 them with the information about what trials are available and
1509 what are the criteria for the patients so that then you could
1510 look at the patient in -- you know, from your community
1511 health center and say here are the options, and evaluate
1512 those right there rather than needing a referral to somewhere
1513 else.

1514 So it is a combination of thinking about the patient and
1515 what trials might be appropriate --

1516 *Mr. Ruiz. Yes.

1517 *Ms. Winckler. -- and making sure that that information
1518 and the ability to do research is there local with the
1519 patient.

1520 *Mr. Ruiz. Yes, you know, currently clinical trials
1521 remain concentrated in a small number of academic medical
1522 centers which slows recruitment, narrows the range of data
1523 that trial sponsors can generate, leaves millions of

1524 patients, particularly those living in rural and underserved
1525 communities, without access to these trials, to these drugs.

1526 Dr. Verst, what can this committee do to encourage
1527 greater participation by community-based providers such as
1528 CHCs in clinical research?

1529 *Dr. Verst. Yes, it is a terrific question, and I do
1530 think it is another one of these multi-pronged approaches, no
1531 silver bullet, per se.

1532 I think ensuring that the community physicians
1533 associated with health care systems are being encouraged and
1534 supported with regard to even referral opportunities. Now,
1535 in order for that to be accomplished, trying to reduce the
1536 burden during this process and, of course, incentivizing as
1537 appropriate, and using technologies that actually -- e-
1538 sourcing, for instance, EMR, and EDC systems being
1539 interconnected to simplify the process and identifying the
1540 right patient for the right clinical trial at the right time
1541 on his or her patient journey.

1542 And of course, this all is working across the health
1543 care ecosystem in order to be a reality.

1544 *Mr. Ruiz. And if I may, just one second, a point of
1545 privilege.

1546 Dr. Hwang, welcome. I went to Harvard Medical School,
1547 did a lot of work at Brigham and Women's Hospital throughout
1548 my years of training, including my fellowship with the

1549 Harvard Humanitarian Initiative. Welcome on board.

1550 *Mr. Griffith. [Presiding.] I thank the gentleman for
1551 yielding back and now recognize Dr. Joyce for his five
1552 minutes of questioning.

1553 *Mr. Joyce. Thank you, Mr. Chairman, for holding this
1554 important hearing and to our panel for being here today.

1555 The United States leads the world in medical innovation.
1556 It is critical that the FDA is operating efficiently to
1557 ensure that patients in the U.S. have access to the best and,
1558 again, the most innovative treatments that are available.

1559 There is now more momentum than ever to innovate in the
1560 chronic disease space. Ongoing research has found better
1561 ways to screen for, diagnose, and delay the onset of symptoms
1562 and diseases like type 1 diabetes. A delayed diagnosis of
1563 T1D drastically increases medical, financial, and emotional
1564 burdens. Frequently, patients go undiagnosed until they land
1565 in the emergency room with the diagnosis of diabetic
1566 ketoacidosis, a life-threatening complication of disease that
1567 can lead to lengthy ICU stays and significant long-term
1568 health consequences. This status quo is bad for patient
1569 outcomes, and it puts unnecessary burdens and costs on the
1570 patient and on our health care system.

1571 Recently Adam Schefter visited Washington, D.C. and
1572 discussed how his wife's late diagnosis of T1D influenced him
1573 to come to Washington to advocate for earlier diagnoses. He

1574 shared with us how, despite an adult diagnosis and early
1575 challenges adapting to the disease, his wife was now able to
1576 successfully manage the disease for over 20 years with the
1577 help of an insulin pump and with the help of continuous
1578 glucose monitoring systems.

1579 To address these issues and to help people more readily
1580 identify and manage the disease before it becomes life-
1581 threatening, I am proud to partner with my colleague and my
1582 friend, Dr. Kim Schrier, on the SCREEN for Type 1 Diabetes
1583 Act. This bill would direct CDC to develop a national
1584 education campaign and provide evidence-based screening
1585 resources to schools, to pediatric practitioners, and to
1586 community health centers.

1587 Dr. Kowalski, what is needed to prevent patients from
1588 reaching deadly symptoms prior to ever being diagnosed with
1589 type 1 diabetes?

1590 *Dr. Kowalski. Thank you for that amazing question,
1591 Representative Joyce. This is such an incredible priority
1592 for us at Breakthrough T1D, and thank you for your
1593 leadership.

1594 I just came from a meeting in Colorado where data was
1595 presented that people who aren't screened for risk, who don't
1596 know they are at risk for type 1 diabetes, have a
1597 approximately 50 percent chance of being admitted in DKA upon
1598 diagnosis, which is diabetic ketoacidosis. So this is a very

1599 dangerous and potentially deadly complication of high blood
1600 sugar and lack of insulin. And in fact, in my home state of
1601 New Jersey we lost a young girl a couple years ago who was
1602 misdiagnosed as having the flu and died because she had DKA.
1603 This is preventable. When screened that risk goes from about
1604 50 percent to less than 4 percent, saving significant money,
1605 potential lives. And we can screen, so we are doing
1606 significant work to make sure that screening happens, that
1607 people are aware and, ideally, that every American is
1608 screened for risk.

1609 *Mr. Joyce. Dr. Kowalski, earlier in your testimony you
1610 said that we in the United States have the opportunity to
1611 become the first country to approve and manufacture a
1612 scalable curative therapy for T1D. What would it take from
1613 the Federal Government to increase screening infrastructure
1614 and ensure that right now that we seize on that opportunity?

1615 *Dr. Kowalski. I think this is a another example. We
1616 keep hearing about the investment in China. We need to
1617 maintain our investment here in the United States, continue
1618 to invest in screening, continue to invest in NIH, continue
1619 to support the -- a fully functional and robust FDA. These
1620 therapies are not a matter of if, they are a matter of when.
1621 And we want them to be when -- soonest here in the United
1622 States.

1623 *Mr. Joyce. In your opinion, the legislation that I am

1624 working with Dr. Kim Schrier, the SCREEN for Type 1 Diabetes
1625 Act, would that help advance screening practices and promote
1626 the early diagnosis of all the goals that we have discussed
1627 here this morning?

1628 *Ms. Winckler. A hundred percent.

1629 *Mr. Joyce. Thank you.

1630 Mr. Chairman. The SCREEN for Type 1 Diabetes is a
1631 strong piece of bipartisan legislation that will have impact
1632 on American lives which will allow for the early diagnosis
1633 and the successful treatment of individuals with type 1
1634 diabetes.

1635 I thank you all for being here today.

1636 Mr. Chairman, I yield the remainder of my time.

1637 *Mr. Griffith. I thank the gentleman. I recognize the
1638 gentlelady from Illinois, Ms. Kelly, for her five minutes.

1639 *Ms. Kelly. Thank you, Chair Griffith and Ranking
1640 Member DeGette, for convening this hearing.

1641 First I would like to acknowledge that this hearing is
1642 being hosted in the wake of the one-year anniversary of the
1643 passage of, in my opinion, the big, ugly bill which has left
1644 already millions of Americans without access to critical
1645 health care. Patients and families across the country count
1646 on our scientific infrastructure to develop lifesaving
1647 treatments, which is why it is so important to invest in
1648 biomedical research while simultaneously supporting access

1649 for those who need it most.

1650 In 2022, along with my colleague, Representative Eshoo,
1651 I pushed forward clinical trial diversity standards at the
1652 FDA, passed through the Food and Drug Omnibus Reform Act.
1653 These standards raised the bar for new FDA applications to
1654 submit a plan to improve the enrollment of clinically
1655 relevant participants from historically under-represented
1656 populations.

1657 The FDA issued proposed guidance for diversity action
1658 plans in June 2024. In January 2025 this Administration
1659 removed the guidance in their harmful attacks on DEI. While
1660 the guidance has been reposted under court order, it has yet
1661 to be finalized, leaving researchers, providers, and patients
1662 in limbo.

1663 Dr. Verst, the IQVIA report, "Advancing Diversity and
1664 Clinical Development Through Cross-Stakeholder Commitment and
1665 Action" highlights -- and I quote -- "failure to include
1666 adequate representation from key subpopulations risks failing
1667 to identify potential differences in therapy, efficacy, or
1668 safety." Can you tell us about the role of diversity in
1669 clinical trials and the safety and efficacy of biomedical
1670 treatment and development?

1671 *Dr. Verst. Thank you. That is a terrific question.
1672 And diversity and representivity is critical for good
1673 science.

1674 And as you mentioned, diversity action plans are
1675 absolutely critical for not only ensuring efficacy and safety
1676 of diverse populations and, of course, increasing probability
1677 of regulatory success, it is obviously very important too to
1678 be concentrating on protocol design, so starting with the end
1679 in mind for that good science.

1680 And that also equates to operationalization of clinical
1681 trials, understanding, for instance, where are these
1682 individuals, patients that are eligible for a specific
1683 protocol. Diverse populations, where are they being treated?
1684 And identifying the right investigators that have the right
1685 patient-eligible populations and even being considerate of
1686 some of the, if you will, nuances associated with even the
1687 eligibility criterion and clearly how they are treated
1688 through the schedule of events, et cetera.

1689 So it is absolutely critical. And in fact, it is good
1690 science.

1691 *Ms. Kelly. Thank you so much.

1692 Dr. Kowalski, can you discuss the importance of clinical
1693 trial diversity for participants with type 1 diabetes?

1694 *Dr. Kowalski. Oh, it doesn't -- type 1 affects all
1695 races and socioeconomic classes, and I am really proud -- a
1696 number of questions have come up on this -- we mandate in our
1697 grant agreements that trials are representative of the broad
1698 population.

1699 Chairman Griffith, you asked about rural areas. We have
1700 seen in federally-qualified health systems -- we have a
1701 program at University Hospital in Newark, for example, that
1702 are having some of the best recruitment for trials. It can
1703 be done. And I think there are mechanisms, especially from
1704 funders and from government, to help push to make sure that
1705 everybody is represented.

1706 I completely agree with my panelists here that we have
1707 to look at all populations to fully understand the impact of
1708 new drugs and new therapies. Often they don't work in every
1709 population and we have to study the broad population to get
1710 that information.

1711 *Ms. Kelly. Thank you so much. And actually, my

1712 district -- I represent the Chicagoland area, but my district
1713 is urban, suburban, and rural, so the south side of Chicago
1714 and then 4,500 farms as you go further south. So rural is
1715 very, very important to me also.

1716 Proper representation in clinical trials ensures that
1717 everyone can be confident that treatments are safe and
1718 effective. As I continue to fight for critical diversity
1719 standards through the NIH Clinical Trial Integrity Act, I
1720 urge the FDA to finalize the diversity action plan guidance
1721 to the standard mandated by the Food and Drug Omnibus Reform
1722 Act of 2022.

1723 And Mr. Chairman, I yield back.

1724 *Mr. Griffith. The gentlelady yields back. I now
1725 recognizes the gentleman from Georgia, Mr. Carter, for his
1726 time.

1727 *Mr. Carter of Georgia. Thank you, Mr. Chairman, and
1728 thank each of you for being here. This is extremely
1729 important and I appreciate all of your expertise here.

1730 You know, I have been around now for 12 years, so I have
1731 seen a lot of what has happened here in the drug industry and
1732 a lot of the policy that has shaped the drug industry in real
1733 time. And it is -- some of it for better and some of it,
1734 unfortunately, for worse.

1735 But, you know, if you go back a couple of decades, we
1736 understand that Europe was really the leader in
1737 biopharmaceutical innovation. And so you ask yourself, what
1738 happened? What happened in Europe, and why did the United
1739 States all of a sudden take over that lead? And one of the
1740 things that happened was the bureaucratic burden and the
1741 price controls that Europe imposed. And we need to learn
1742 some important lessons from that, not to let that happen here
1743 in America.

1744 I often say that I practiced pharmacy for over 40 years
1745 and I saw nothing short of miracles as a result of research
1746 and development, and we want to continue with that and we
1747 want to continue to make sure that companies are -- that we
1748 are letting the markets work, and that we are letting capital

1749 flow to risk, and that we are letting science lead. That is
1750 extremely important.

1751 I do understand and appreciate the point that has been
1752 made here about China and about them taking the lead. But we
1753 can't beat China by copying China because China does not
1754 operate under the same rules and regulations that we do. We
1755 all understand that. We are going to beat them the same way
1756 that we beat Europe, and that is by doubling down on what
1757 actually made America the leader in the first place:
1758 entrepreneurial spirit, access to capital, free markets, and
1759 the best science in the world. And I still say we have the
1760 best scientists in the world right here in the United States
1761 of America.

1762 Dr. Verst, I want to ask you first. From where you sit,
1763 what are the one or two pro-innovation policies that Congress
1764 should be prioritizing right now to make sure that the U.S.
1765 holds our lead instead of policies that risk ceding our
1766 leadership to China?

1767 *Dr. Verst. Terrific question. I think that most
1768 recently, of course, Trialblazer, the Operation Trialblazer,
1769 those components, I think, are critical insomuch that it does
1770 ensure the coalescence or, if you will, all of the HHS
1771 divisions coming together.

1772 We talked here today about, of course, the FDA. And I
1773 know it is not within the scope, but that adjacency will be

1774 critical, especially -- for instance we talked about today
1775 community-based practitioners becoming more integral into
1776 clinical trials here in the U.S. And I will just give a few
1777 data points.

1778 Oncology. Oncology is the world's largest clinical
1779 trial market. Over 40 to 50 percent of the entire global
1780 clinical trials resides within oncology. Our oncology
1781 clinical trial investigator capacity is being taxed,
1782 especially, as stated, more of the academic medical
1783 centers --

1784 *Mr. Carter of Georgia. Right.

1785 *Dr. Verst. It is concentrated there. We have to get
1786 out into the communities, you know, so that -- these are, I
1787 think, important components that will help us to ready
1788 ourselves for the volume of clinical trials and, of course,
1789 the resulting capacity in order to accommodate them.

1790 *Mr. Carter of Georgia. Okay. Well, let's do kind of
1791 shift gears a little bit and talk about the clinical trials
1792 because we talked about it a little bit up here, about
1793 decentralizing it, and that is something that I really do
1794 think is -- can be beneficial, particularly in using all the
1795 health care facilities that are available.

1796 It was mentioned, FQHCs. I would also carry that to be
1797 local pharmacies. That is another area we can -- remember,
1798 pharmacists are the most accessible health care professionals

1799 in America. Over 90 percent of all Americans live within 5
1800 miles of a pharmacy, so we need to take advantage of those
1801 type of things.

1802 FDA's own guidance recognizes that fully decentralized
1803 approaches aren't right for every trial, and I understand
1804 that. But Ms. Winckler, I want to ask you, what is standing
1805 in the way of scaling decentralizing trials in the U.S.
1806 today, and what can FDA and Congress do to remove those
1807 barriers?

1808 *Ms. Winckler. So we have talked about some of the
1809 barriers, and some of it is training those local
1810 investigators. Some of it is even just asking not only the
1811 patient, but asking those providers who might be willing to
1812 participate, so the community pharmacist or the community
1813 health centers.

1814 And then there is a basic problem which is contract and
1815 budget negotiation. It wastes time and it is -- it happens
1816 again and again. And it may seem strikingly basic and not
1817 worth the time of addressing, but it stops engaging new
1818 investigators and it stops patients who want to participate
1819 in trials having the opportunity to do so. We have to fix
1820 it.

1821 *Mr. Carter of Georgia. Well, that is -- thank you for
1822 mentioning that. It never occurred to me. I didn't realize
1823 that was such a problem.

1824 Okay, well, thank you all very much, and I yield back.

1825 *Mr. Griffith. The gentleman yields back. I now

1826 recognizes the gentlelady from Michigan, Mrs. Dingell, for
1827 her five minutes of questioning.

1828 *Mrs. Dingell. Thank you, Mr. Chair. Thank you for
1829 holding this hearing on the importance of maintaining our
1830 leadership in drug development.

1831 Few people understand the need for a robust, safe, and
1832 efficient drug development process like patients with rare
1833 diseases and their families. As a result of the Orphan Drug
1834 Act there have been important advances in research on and
1835 treatment for rare diseases. Unfortunately, the Trump
1836 Administration's actions have risked rolling back that
1837 progress. The scientists and researchers at the National
1838 Institute of Health who would have previously received
1839 funding to develop innovative, lifesaving treatments for rare
1840 diseases are losing access to grant funding and heading to
1841 other countries with more reliable opportunities: a serious
1842 issue I think many people don't understand.

1843 These impacts are hitting real people firsthand. At the
1844 University of Michigan faculty staff -- most importantly, the
1845 students -- are telling me we are going to lose the next
1846 generation of researchers. There are kids that I say, can't
1847 you wait? It will get stable. And they are, "We are not
1848 going to be a ping pong ball. We have to have a reliable

1849 income.' ' And I think that is dangerous for this country,
1850 that we are going to lose the next generation of researchers
1851 and that this instability is impacting their work towards
1852 real lifesaving, innovative, and breakthrough discoveries.

1853 Cuts to the FDA workforce have added to this chaos,
1854 slowing review timelines and making it more difficult for
1855 drug-makers and patients to get answers about the approval
1856 process for treatments that could bring hope to the rare
1857 disease community. Dr. Kowalski, I have heard from several
1858 companies and patient groups that the turmoil at FDA has
1859 impacted the drug approval process. Have you heard that
1860 companies are concerned with the instability of that agency,
1861 and that they are indicating that they have to think about
1862 contingency planning?

1863 *Dr. Kowalski. Yes, we have. I -- this is a concern
1864 for companies that are working in diabetes, American
1865 companies who have what we think will be curative therapies.
1866 They are proceeding at FDA, but we have heard they are making
1867 contingency plans. This includes Canada. This includes
1868 Europe, Australia. And that is concerning to us.

1869 *Mrs. Dingell. Dr. Kowalski, how will drastic cuts to
1870 federally-funded health research, including this recent
1871 proposed rule that -- I will behave and not totally go forth
1872 on -- to devalue peer review and politicize the Federal
1873 grant-making process affect our ability to advance new

1874 therapies, including for rare disease treatments?

1875 *Dr. Kowalski. I often say it takes a village to move a
1876 life-changing breakthrough to a person and have their life be
1877 changed. I think sometimes we think that this is all the
1878 responsibility of private companies. Virtually every
1879 advancement in type 1 diabetes originated with a special
1880 diabetes program, including pivotal FDA trials. Teplizumab,
1881 a disease-modifying therapy, closed loop systems, the NIH
1882 funded pivotal trials. Companies then drove these therapies
1883 to the market. People have done better. So the public-
1884 private partnership is incredibly important here in the
1885 United States, and I am proud of the role some of our funding
1886 has played. It takes a lot to get, but cutting funding would
1887 be incredibly short-sighted.

1888 *Mrs. Dingell. Thank you.

1889 Mr. Bollyky, what -- this is what really worries me,
1890 too. What will these funding cuts mean for U.S.
1891 competitiveness with other countries and our adversaries,
1892 including China?

1893 Could we see more researchers leave the U.S. for more
1894 stable funding environments abroad?

1895 And I am told -- you know, after World War II Germany
1896 led, we led. I am told now we are number two in research on
1897 -- versus China. Could you comment?

1898 *Mr. Bollyky. Absolutely, great question. We are

1899 already seeing a flow of scientists out of the U.S. That is
1900 a product of the difficulty of the U.S. funding environment
1901 as well as the rise or emergence of China's biopharmaceutical
1902 industry. So this is a real risk.

1903 It is also a risk at the agency level. The reality is
1904 some of why it is hard to go through the IND process is due
1905 to unpredictability and inconsistency from FDA staff. We
1906 have lost staffers, we have lost a lot of expertise and
1907 experience, and that is affecting our competitiveness as
1908 well.

1909 *Mrs. Dingell. I have 15 seconds. Do you think this is
1910 a national security issue?

1911 *Mr. Bollyky. It is absolutely a national security
1912 issue.

1913 *Mrs. Dingell. Thank you.

1914 I will yield back, Mr. Chair, and thank you to all the
1915 witnesses.

1916 *Mr. Griffith. The gentlelady yields back. I now
1917 recognize the gentlelady from Florida, Mrs. Cammack, for her
1918 five minutes of questioning.

1919 *Mrs. Cammack. Thank you, Mr. Chairman. Thank you to
1920 our witnesses for being here today.

1921 So obviously, there is a little bit of a dispute in who
1922 is leading, but I don't think at this point we are going to
1923 argue about that.

1924 But talk about the importance of this leadership and
1925 maintaining dominance in this space. That means that we not

1926 only accelerate the development of new therapies, but
1927 ensuring that we have a regulatory framework that inspires
1928 confidence, that strengthens our domestic research ecosystem
1929 and expands opportunities for Americans to participate in
1930 clinical trials. And as the representative covering the Sid
1931 Martin Biotech hub, a world-renowned facility, this is
1932 critically important not just to our national security, but
1933 to folks for me back home. So I am going to start with you,
1934 Mr. Bollyky. Did I say that right? Yes, all right.

1935 So clinical development is increasingly global, as been
1936 highlighted here today. But the United States can remain a
1937 leader in biomedical innovation by strengthening our domestic
1938 research ecosystem while continuing to build on those trusted
1939 partnerships with countries that share our regulatory
1940 standards and commitment to high-quality science.

1941 At the same time, FDA has made it clear that it can rely
1942 on foreign clinical data only when it has confidence in the
1943 integrity of that data. As more early-stage development
1944 takes place around the world, that confidence depends on FDA
1945 having the resources to inspect the foreign trial sites and
1946 verify compliance with good clinical practice standards,
1947 something I don't have the confidence in right now.

1948 As Congress looks toward next user fee reauthorization,

1949 should we be thinking about those objectives together,
1950 continuing to build on trusted partnerships while
1951 strengthening FDA's foreign inspection capacity, and creating
1952 incentives to keep more of that early-stage drug development
1953 here in the United States?

1954 And specifically and quickly, what reforms would you
1955 recommend?

1956 *Mr. Bollyky. Terrific question. Thank you for it.

1957 The FDA adopts a risk-based standard to conducting its
1958 good clinical practice inspections. It is hard to square a
1959 risk-based approach with where those inspections are
1960 happening currently.

1961 China -- the number of trials or inspections we are
1962 conducting in China is not only is dwarfed by the number of
1963 inspections we do in the U.S.; it is also true for Japan and
1964 Europe. China is -- has a number -- fewer inspections in
1965 that environment.

1966 Congress should consider instituting a user fee for INDs
1967 that involve foreign clinical trial data so that FDA can have
1968 more resources to be able to pursue these inspections. We
1969 saw something similar in the manufacturing space around
1970 generic drug manufacturing and it helped uncover concerns
1971 with good manufacturing practices. The same would be true
1972 for good clinical practices.

1973 *Mrs. Cammack. And you would agree that we need to

1974 continue to develop the incentives for more early-stage drug
1975 development here domestically?

1976 *Mr. Bollyky. Absolutely. It really does need to be a
1977 combination of making the U.S. competitive and also having
1978 better oversight over the activity occurring in China.

1979 *Mrs. Cammack. I 1,000 percent agree. Thank you.

1980 Dr. Winckler, I would like to shift to another
1981 opportunity for reform where -- there you are. So women have
1982 historically been under-represented in many clinical trials,
1983 if not most, particularly where there are therapies that are
1984 ultimately intended for both men and women. So while we have
1985 seen some progress, it is not near enough. I want to look
1986 towards how -- the next user fee reauthorization and how
1987 Congress could specifically be thinking about increasing
1988 enrollment, but ensuring that sponsors are designing trials
1989 from the outset to generate meaningful sex-specific safety
1990 and efficacy data. Can you speak to that?

1991 *Ms. Winckler. Yes, so there are a couple of
1992 opportunities. First it is making sure that the sponsors are
1993 thinking about what are the endpoints that are relevant to
1994 women or -- shall we say are there endpoints that are
1995 different by sex, and making sure that that voice of the
1996 individual is heard in designing it. And then, in looking at
1997 recruiting for trials and engaging in the research, what are
1998 the barriers and what sex-based differences need to be

1999 explored not only scientifically, but practically in getting
2000 then the right mix of participants in the clinical trial.

2001 So it requires intentionality and then also looking at
2002 things -- are there opportunities to explore some of these
2003 post-market, right? What needs to be done pre-marketing, and
2004 then what might be explored and better understood after a
2005 product is already on the market. So using real-world data
2006 to generate real-world evidence to help us tease out sex-
2007 based differences.

2008 *Mrs. Cammack. Well, and I know I only have 13 seconds,
2009 but how do you do that without creating additional burdens on
2010 the sponsors?

2011 *Ms. Winckler. So I think this is part of the up front
2012 and being clear. Where you have particular sponsor burden is
2013 when it comes in late or there is a lack of clarity or --
2014 sometimes sponsors don't always listen perhaps as clearly as
2015 they should have up front.

2016 *Mrs. Cammack. I appreciate that. Thank you.

2017 *Mr. Griffith. The gentlelady --

2018 *Mrs. Cammack. And I will submit the rest of my
2019 questions for the record.

2020 [The information follows:]

2021

2022 *****COMMITTEE INSERT*****

2023

2024 *Mr. Griffith. The gentlelady yields back.

2025 *Mrs. Cammack. I yield.

2026 *Mr. Griffith. And I now recognize the gentlelady from
2027 California, Ms. Barragan.

2028 *Ms. Barragan. Thank you, Mr. Chairman.

2029 Mr. Bollyky, I want to start with you.

2030 [Chart]

2031 *Ms. Barragan. I have this chart that I used in July of
2032 2025. It is an FDA organizational chart. I don't know if
2033 you could see it from where you are, but basically what it is
2034 is it has the different sections of FDA. And when I use this
2035 -- actually, this is now gone. We have got four more gone.
2036 This is now gone. The we have got four total.

2037 But one of them that has been gone I want to point out
2038 is the food oversight. And why am I bringing up the food
2039 oversight? Because I am getting calls in my congressional
2040 office about this concern right now about this explosive
2041 diarrhea-causing illness calls called cyclosporiasis -- I
2042 don't know if I have got that right -- which is like going
2043 through thousands -- it is going through a number of states,
2044 and thousands of people are dealing with it.

2045 Do you think that the elimination of these offices
2046 within the FDA is a good thing?

2047 *Mr. Bollyky. For both the conversation we are having
2048 around clinical trial oversight and attracting more activity,

2049 as well as ensuring public health in the U.S. we need a
2050 robust FDA appropriately staffed to do its job.

2051 *Ms. Barragan. Okay. So, you know, right around the
2052 same time that I was using this chart you retweeted this
2053 article called, "Inside the Collapse of the FDA" from The
2054 New York Times. Would you agree that it is important that we
2055 have experienced voices and institutional knowledge at the
2056 FDA?

2057 *Mr. Bollyky. If we are going to compete with other
2058 countries, especially China in biomedical innovation, we need
2059 to have the expertise and the experience to do the best
2060 regulatory science at the FDA.

2061 *Ms. Barragan. And do you agree that we need to protect
2062 the U.S. system of biomedical innovation?

2063 *Mr. Bollyky. Absolutely. It is a priority for
2064 biosecurity, for public health, and for patients.

2065 *Ms. Barragan. And would we help achieve that goal if
2066 we are making cuts to the National Institutes of Health?

2067 *Mr. Bollyky. One of the foundations to the U.S.
2068 biomedical innovation system is robust funding for basic
2069 research from the NIH.

2070 *Ms. Barragan. So it would not help if we are cutting
2071 NIH.

2072 *Mr. Bollyky. Cutting NIH funding does not advance our
2073 ability to have that basic research that has informed so much

2074 of U.S. drug development.

2075 *Ms. Barragan. Okay. And so if we are trying to

2076 protect the U.S. system of biomedical innovation, does it

2077 help if we are having and continue to have hostility to

2078 international students from abroad?

2079 *Mr. Bollyky. We need to have an environment that

2080 attracts the best scientists both domestically and

2081 internationally to be able to compete in the current global

2082 environment.

2083 *Ms. Barragan. Okay. So it doesn't help that that is

2084 happening.

2085 And what about -- does it help us be the leaders in

2086 biomedical innovation if the United States is attacking

2087 research institutions here?

2088 *Mr. Bollyky. Again, the foundation for U.S.

2089 biomedical innovation has really been the universities and

2090 our basic research. We need to support them.

2091 *Ms. Barragan. Right. So it is not helping. And Mr.

2092 Bollyky, I think you know what I am talking about. You wrote

2093 an article in which you specifically said -- and now I am

2094 going to just quote from it if I could find the sentence

2095 about -- "The U.S. has led the pharmaceutical industry

2096 because of collaboration among American universities.'" And

2097 you go on to say more things. "Yet this resource is not

2098 inexhaustible. The President risks squandering it through

2099 his cuts in funding for the National Institutes of Health,
2100 hostility to international students, and attacks on research
2101 universities.'` And that is why I am asking you, because you
2102 wrote about this specific thing.

2103 Let me ask you another thing about -- because this
2104 hearing is about biomedical innovation, and it is interesting
2105 that it says "the role of the FDA'" when we are gutting the
2106 FDA and the FDA is collapsing. How about placing blanket
2107 tariffs on pharmaceuticals? Is that going to help the U.S.
2108 be less dependent on foreign drugs?

2109 *Mr. Bollyky. Tariffs, if deployed, need to target
2110 where that dependance arises, and that is really on upstream
2111 inputs particularly from China. So if we are going to use
2112 tariffs, they need to appropriately target that level of
2113 ingredients and they need to exempt allied and well-
2114 regulated, diversified sources.

2115 *Ms. Barragan. And yet again, this Administration is
2116 doing that and the Republican Congress is totally supporting
2117 that.

2118 I am setting -- your article of July 22, 2025 called
2119 "America's Pill Problem,'" and its subheading is "Tariffs
2120 Won't Fix the Country's Reliance on Foreign Medicines.'" So
2121 here we have all these actions being taken by this
2122 Administration, all these actions supported by this
2123 Republican Congress, and we are having a hearing about how to

2124 actually be leaders. Yet they are not speaking out against
2125 these particular actions. Mr. Bollyky, thank you.

2126 And I yield back.

2127 *Mr. Griffith. The gentlelady yields back. I now
2128 recognize the gentleman from New Jersey, Mr. Kean.

2129 *Mr. Kean. Thank you, Mr. Chairman, and I appreciate

2130 the committee beginning the important preparation to
2131 reauthorize the prescription drug user fee amendments next
2132 year.

2133 New Jersey boasts a thriving biotechnology industry, and
2134 not only provides jobs locally but provides the world with
2135 therapies that improve and extend life. Ms. Verst and Ms.
2136 Winckler, my district is home to numerous small rare disease
2137 companies. These constituents and other companies have
2138 frequently raised concerns about inconsistency, and even
2139 within FDA review divisions. This is a significant source of
2140 uncertainty that translates to an unpredictable regulatory
2141 environment and disincentives investment in areas of unique
2142 need. What additional steps can the FDA take to improve
2143 consistency among FDA reviews of rare disease products while
2144 also preserving an adaptive framework that acknowledges the
2145 unique challenges of rare disease drug development?

2146 Both of you.

2147 *Ms. Winckler. Yes, so thank you, and there are a
2148 couple of ways.

2149 One, I will say that the FDA's Rare Disease Innovation
2150 Hub has actually been quite helpful in bringing together not
2151 only the different product centers at FDA -- so Center for
2152 Biologics, Center for Drugs, and Center for Devices -- and
2153 stimulating the conversation so that they can have a better
2154 understanding of what endpoint might be relevant in a rare
2155 disease and how to use that endpoint, but there are further
2156 opportunities. It comes from greater collaboration and
2157 conversation with the patient communities to make sure that
2158 the end points that are being pursued are patient-relevant,
2159 but then also conversation among the divisions at FDA and
2160 explanation where they may diverge.

2161 There sometimes are scientific rationales that what is
2162 good enough for one division in affecting one symptom or
2163 characteristic of a rare disease is appropriate, and a
2164 different review division may have a different standard but
2165 it is scientifically justified. If we don't know that
2166 scientific justification, it looks wrong or unfair. And so
2167 learning more and sharing more, so having more of those
2168 conversations and sharing the learnings in rare disease, I
2169 think, is one of the greatest opportunities we have.

2170 *Mr. Kean. Thank you.

2171 Ms. Verst?

2172 *Dr. Verst. Terrific question. I think several ways
2173 the agency can help on the rare disease front.

2174 Notably, over 65 percent of all clinical trials
2175 conducted around the globe, phase one through three, are
2176 coming from biotech customers. Biotech customers need more
2177 certainty. And of course, their investors need more
2178 certainty to fund their rare disease clinical trials.

2179 I think that, having said that, providing greater
2180 clarity and guidance will be very important to try to reduce
2181 some of the uncertainty, and I do think that the Operation
2182 Trialblazer addresses many of this in terms of, for instance,
2183 highlighting the use of, you know, for instance, one adequate
2184 and well-controlled trial with confirmatory evidence. You
2185 know, that is a really important step forward, and that
2186 revised draft guidance will help to provide just that more
2187 certainty. Thank you.

2188 *Mr. Kean. Yes, thank you.

2189 Ms. Winckler, in your testimony you stressed the
2190 importance of doing more with less, which is critical for the
2191 development of oncology and rare disease products where
2192 trials are costly and it is difficult to find patients.

2193 First, how can the FDA support modernizing clinical
2194 trial designs and structures to allow for more patient access
2195 to trials?

2196 *Ms. Winckler. There are a couple of different
2197 mechanisms. Part of it is clarity on the part of the
2198 regulator. We have said repeatedly this morning that it is

2199 easier to follow rules that you have heard about and
2200 understand and can ask questions. So part of it is the
2201 clarity from the regulator and then, in particular, clarity
2202 about this idea of a hub and spoke model, where I may be
2203 responsible at a central location for the research oversight,
2204 then it makes it easier to extend the clinical trial
2205 expertise and reach.

2206 *Mr. Kean. Okay. And second, how can Congress
2207 strengthen FDA's ability in this through the user fee
2208 reauthorization next year?

2209 *Ms. Winckler. So as part of the user fee
2210 reauthorization process, you know, there will be important
2211 situations emerge where actually revising the statute would
2212 be helpful, and I think that that will -- there were some
2213 ideas today and others that will emerge.

2214 And then tailoring the resources that are needed to
2215 implement some of those ideas. A statutory requirement for
2216 foreign inspections, for example, without any at least
2217 assessment of the resources necessary is a requirement that
2218 is just logically unlikely to be met.

2219 *Mr. Kean. Okay, thank you.

2220 Ms. Verst, there are, unfortunately, many barriers to
2221 mental health care, and I am concerned -- no, excuse me --
2222 and I am committed to eliminating those barriers. One of
2223 those barriers is limited development of psychiatric drugs

2224 compared to drugs to treat physical health conditions.

2225 First, can you speak to the unique barriers faced by
2226 psychiatric drug sponsors?

2227 *Dr. Verst. Yes, another terrific question.

2228 I think some of the barriers really is patient access
2229 and, of course, that being hugely important when conducting
2230 any clinical trial, but especially psychiatric trials. And
2231 so promoting more of the community-based practitioners --
2232 because clearly, academic medical centers alone will not help
2233 solve this problem. So increasing awareness with -- and
2234 within the community, the practicing physician community in
2235 particular, and really taking on many of the areas of
2236 opportunity that Susan has mentioned here today would be
2237 extremely helpful in accessing and recruiting those patients
2238 into clinical trials and, of course, understanding their
2239 needs and requirements to operationalize the clinical trials
2240 with their needs in mind to retain those patients and, of
2241 course, extracting the necessary efficacy and safety data to
2242 be promulgating new medications for these patients.

2243 *Mr. Kean. Okay. And second, what recommendations
2244 would you have --

2245 *Mr. Griffith. I hate to interrupt you, but we will
2246 have to do questions for the record.

2247 *Mr. Kean. Okay.

2248 *Mr. Griffith. You are way over time.

2249 *Mr. Kean. All right. Well, thank you then. I yield

2250 back, and I will follow up with the follow-up questions

2251 regarding what we can do in Congress.

2252 [The information follows:]

2253

2254 *****COMMITTEE INSERT*****

2255

2256 *Mr. Griffith. I appreciate it.

2257 *Mr. Kean. I will follow up.

2258 *Mr. Griffith. Thank you.

2259 *Mr. Kean. Thank you, Mr. Chairman.

2260 *Mr. Griffith. I now recognize the gentlelady from New
2261 York, Ms. Ocasio-Cortez.

2262 *Ms. Ocasio-Cortez. Thank you, Mr. Chairman, and thank
2263 you to all of our witnesses for being here today.

2264 I want to talk about one aspect of innovation in health
2265 care, which is our pharmaceutical patent system. I have
2266 served on many different committees in Congress at this
2267 point: Financial Services, you know, Natural Resources,
2268 Oversight. And this word, "innovation," can include really
2269 incredible breakthroughs that save people's lives and also a
2270 lot of scammy behavior that we see across industries, you
2271 know, in financial services -- stock buybacks, for example,
2272 being an innovation. That doesn't really create anything new
2273 of value, but it is certainly a new way to juice some
2274 numbers.

2275 And so I think I want to dig in a little bit on our
2276 pharmaceutical patent system because this system is critical
2277 to drug development in the United States and ensuring that
2278 breakthrough drugs become available to patients here. But it
2279 is also a system that is abused by big pharma to prevent
2280 longstanding drugs from becoming cheaper and more widely

2281 available to everyday Americans.

2282 Dr. Hwang, when it comes to pharmaceuticals the Federal
2283 Government reviews a company's patent application and
2284 determines whether or not it meets specific standards like
2285 creating a brand new drug to treat a specific disease. Is
2286 that correct?

2287 *Dr. Hwang. That is correct.

2288 *Ms. Ocasio-Cortez. And when patents -- and these
2289 patents, when a drug company earns a patent for a new drug,
2290 they get a period of protection from competition when no
2291 other company can sell that same drug. Typically 20 years
2292 from the filing, correct?

2293 *Dr. Hwang. Right.

2294 *Ms. Ocasio-Cortez. And the logic of this is that if
2295 you are a company and you invested millions of dollars in the
2296 development of a drug, you should have exclusivity once you
2297 develop that drug to earn that money back and have that
2298 return and be rewarded for that innovation, right? That is
2299 kind of the gist of the system.

2300 But over the years we have seen some drug companies do
2301 some curious things with this. We have actually seen them, I
2302 think, abuse this period of protection to create monopolies
2303 over the market and then increase the prices on their drugs.
2304 One example of big pharma abusing this system is when they
2305 file additional patents they will make a new drug, they will

2306 get their 20 years, then they will file additional patents
2307 that involve really minor and often unnecessary changes to
2308 their products. So they will have a great pill and then they
2309 will just change it to a capsule and then refile the patent.
2310 And we are seeing that in quite a few treatments, correct?

2311 *Dr. Hwang. That is correct. And in a small number of
2312 cases some of these reformulations can help our patients.
2313 They can make treatments more available and easier to use.
2314 But in the vast majority of cases, these are life cycle
2315 extending.

2316 *Ms. Ocasio-Cortez. Yes. In fact, the pharmaceutical
2317 company AbbVie used this strategy with their drug Humira
2318 which is used to treat rheumatoid arthritis. By barely
2319 changing their products they obtained over 130 patents that
2320 gave them continued exclusive market control over rheumatoid
2321 arthritis treatments. And during this time the price from
2322 Humira went from \$500 for one syringe to nearly \$3,000 per
2323 syringe, and that led to about \$80,000 for a 1-year supply
2324 for a patient.

2325 In fact, AbbVie isn't the only one doing this. I have
2326 an example right here from AstraZeneca. This is an inhaler
2327 pump. It was loaned to me by someone with asthma. And we
2328 have seen that AstraZeneca, what they did was that they had
2329 this treatment. The actual medication inside did not change
2330 at all, the administration of it, but they filed a new patent

2331 to maintain exclusivity of it. And can you guess what was
2332 the great innovation that was worth this not going generic
2333 for?

2334 *Dr. Hwang. I am not aware, but I think you know the
2335 answer, Congresswoman.

2336 [Laughter.]

2337 *Ms. Ocasio-Cortez. Yes. It is this little plastic
2338 piece right here, and it is one of the big features of that
2339 new patent. This thing to keep the cap from coming off was a
2340 major part of the new patent that they had filed and -- to
2341 extend and to prevent this lifesaving drug from going generic
2342 and bringing down costs for everybody.

2343 I was just curious. What do you think we do about this?

2344 *Dr. Hwang. So I think very quickly -- and I would be
2345 happy to submit more for the record -- but these strategies,
2346 unfortunately, reward the very thing that we should be
2347 avoiding, which is aggressive legal maneuvers instead of the
2348 risky basic science that our country should prioritize.

2349 *Ms. Ocasio-Cortez. Great. Thank you very much.

2350 And I yield back.

2351 *Mr. Griffith. The gentlelady yields back. I now
2352 recognize the gentleman from Ohio, Mr. Balderson, for his
2353 five minutes of questioning.

2354 *Mr. Balderson. Thank you, Mr. Chairman, and thank you
2355 all for being here. My first question is for Mr. -- Dr.

2356 Kowalski.

2357 Thank you for being here. Your testimony mentions how
2358 the breakthroughs that wearables such as continuous glucose
2359 monitoring devices have helped millions of patients manage
2360 their diabetes, and that these were made possible due to
2361 sustained research, investments, and a strong FDA review
2362 process. What ideas do you have to improve clinical trial
2363 designs and requirements to spur innovation for these types
2364 of wearable technologies to continue to help patients with
2365 type 1 diabetes?

2366 *Dr. Kowalski. Thank you for that question. It is a
2367 real point of pride that I got to work on the project that
2368 developed many of these therapies, and my brother and I
2369 benefit, as do many, many people, which is amazing.

2370 I think what we saw in that process and what we need to
2371 continue is clear -- and we have heard this from a number of
2372 the panel members -- clear and consistent regulation. We
2373 want sponsors to know what they need to do.

2374 I think we need to continue to have the voice of the
2375 patient at the table. You know, as we continue to innovate,
2376 type 1 diabetes isn't solved, as are many diseases. We still
2377 have a lot of work to do. And the risk benefit -- we need
2378 the voice of the patient and the voice of the clinicians at
2379 the table.

2380 We also need to accelerate the use of more biomarkers.

2381 You know, as we look at how fast can we move the trials, for
2382 example, in some of the -- even next-gen type 1 diabetes
2383 therapies, we have been limited by an old way to look at type
2384 1 diabetes that is not even applicable when you are at risk
2385 for the disease, which has really slowed down progress. So
2386 better bio markers.

2387 And I just can't stress enough the risk benefit
2388 barriers. Being risk averse when people are suffering,
2389 developing diabetic complications, living with this grueling
2390 disease, we need to take that into consideration and continue
2391 to innovate.

2392 *Mr. Balderson. Thank you very much, and I appreciate
2393 your passion with that. So my next question is for Dr.
2394 Verst.

2395 Thank you for being here, ma'am. I often hear that rare
2396 disease drug development is limited not by scientific
2397 opportunity, but by the difficulty of identifying, enrolling,
2398 and retaining patients in clinical trials. As we look
2399 forward for ways to strengthen America's leadership in
2400 biomedical innovation, what specific regulatory or policy
2401 barriers still prevent broader participation in rare disease
2402 clinical trials?

2403 And what actions should Congress take to ensure that
2404 rare disease patients can access innovation research,
2405 regardless of where they live?

2406 *Dr. Verst. Terrific questions.

2407 I think, initially, patient access and understanding
2408 where -- these patients, their standard of care, the patient
2409 journeys are just critical questions to help us when
2410 identifying rare disease trial opportunities, trying to
2411 access those patients at the right time on their journey.
2412 And that could be achieved through real-world evidence and
2413 understanding the natural history of their diseases as well.

2414 And insomuch as using that real-world data and, of
2415 course, ensuring that we are helping with the design of
2416 clinical trials that is very simplified and not burdensome
2417 for neither the patient nor their investigator, and that, I
2418 think, is clearly of importance. And where you can help --
2419 and of course, again I refer back to project operation, the
2420 Operation Trialblazer, where there is some clear guidance
2421 that you can help us to ensure that draft and revised
2422 guidance on, for instance, one randomized, well-controlled
2423 trial with confirmatory evidence, that helps to reduce the
2424 number of patients required for the generation of evidence,
2425 and that being so critical to ensure that treatments are
2426 getting -- better treatments to our patients faster.

2427 *Mr. Balderson. Okay, thank you. I will stay with you,
2428 Doctor. Clinical trials produced plenty of data, but
2429 sponsors frequently report that the process of gathering and
2430 submitting this information is still unnecessarily

2431 complicated. Where are there opportunities for FDA to
2432 leverage digital technologies to reduce administrative
2433 burden, improve trial efficiencies, and allow researchers to
2434 spend more time developing therapies and less time navigating
2435 paperwork?

2436 You have about 20 seconds, ma'am, I apologize.

2437 *Dr. Verst. Okay. I am going to go very fast.

2438 *Mr. Balderson. Thank you.

2439 *Dr. Verst. Firstly, I think with regard to the use of,
2440 again, real-world evidence, life science models, you
2441 mentioned digital twins, novel trial designs which is
2442 absolutely critical.

2443 In addition, you mentioned wearables, the use of
2444 patient-reported outcomes, digital-connected devices. And
2445 reducing, if you will, the burden on the investigators and
2446 patients is just incredibly important. And the good news is
2447 that the -- those digital data sets actually reduce less
2448 time, less burden, less cost in cleaning data. And in fact,
2449 those data can actually be surfaced for early signal
2450 detection, thus accelerating development.

2451 *Mr. Balderson. Ma'am, thank you very much.

2452 Mr. Chairman, I yield back.

2453 *Mr. Griffith. The gentleman yields back. Now I
2454 recognize the gentleman from Louisiana, Mr. Carter, for his
2455 five minutes.

2456 *Mr. Carter of Louisiana. Thank you, Mr. Chairman and
2457 Ranking Member, and to our witnesses for joining us today.

2458 We are here today to discuss the critical role of FDA
2459 and the importance of maintaining America's leading
2460 leadership in scientific research and innovation on a global
2461 scale, yet Republicans and the Trump Administration have
2462 spent the last year attacking the Federal workforce while
2463 dismantling and politicizing the research that drives the
2464 development of lifesaving treatments and cures.

2465 Now, to make matters worse, the Office of Management and
2466 Budget is proposing some of the most sweeping and damaging
2467 changes to the Federal grant-making system in modern history.
2468 These reforms will replace longstanding, non-partisan, merit-
2469 based grant-making processes with ones that is subject to
2470 political influence and control.

2471 Federally-funded research is the infrastructure that
2472 powers American innovation, science, and national security.
2473 When we cut that funding, we aren't just trimming the budget
2474 line; we are shrinking the pipeline of scientists and
2475 researchers that this country depends on to remain
2476 competitive on a global scale. At a time when our foreign
2477 adversaries are strategically making investments in
2478 scientific education and emerging technologies, choosing to
2479 shrink our own talent pipeline isn't fiscally responsible; it
2480 is a big mistake. The consequence of these actions will be

2481 felt for decades and weaken our ability to compete and
2482 innovate and lead globally.

2483 Dr. Hwang, I proudly represent New Orleans, home of
2484 world-class research institutions like Tulane University, LSU
2485 Health, and many others. These institutions rely on Federal
2486 funding to advocate groundbreaking biomedical research,
2487 innovation, and develop discoveries to improve lives and
2488 health outcomes. How do Federal research funding cuts affect
2489 the United States' leadership in biomedical research and
2490 innovation?

2491 *Dr. Hwang. Cuts to that Federal investment, as you
2492 mentioned, Congressman, are tremendously harmful, and they
2493 threaten to push back all the leadership that we have gained
2494 over the years.

2495 *Mr. Carter of Louisiana. From your experience as a
2496 physician and an academic medical center, what impact can
2497 these disruptions have on clinical trials and patient access?

2498 *Dr. Hwang. It could have a harmful role, for sure, on
2499 the initiation of new trials, and it also creates a freeze
2500 over trials that are already in progress.

2501 And I just want to stress for the record that these
2502 views are my own and don't represent those of my employer.

2503 *Mr. Carter of Louisiana. Fair enough. How do these
2504 cuts undermine the global competitiveness, particularly
2505 competition with countries like China?

2506 *Dr. Hwang. Again, I think loss of investment in
2507 American science does a big disservice to our efforts to
2508 create new therapies.

2509 *Mr. Carter of Louisiana. Why is maintaining a strong
2510 workforce at FDA important to advancing new treatments and
2511 protecting patients?

2512 *Dr. Hwang. As we have heard from other members of this
2513 panel, the FDA has an incredible role in the innovation
2514 ecosystem, and our leadership over the past two decades
2515 relies on a well-functioning and well-staffed FDA.

2516 *Mr. Carter of Louisiana. You have got Members of
2517 Congress sitting here with you. What should we do? If you
2518 now have a magic wand and you are talking to Members of
2519 Congress who serve on this powerful subcommittee, how would
2520 you use your magic wand to tell us to do better?

2521 *Dr. Hwang. Representative, first I would start by
2522 asking how much time you have.

2523 [Laughter.]

2524 *Mr. Carter of Louisiana. I have a minute and 12 so you
2525 can give me a good answer.

2526 [Laughter.]

2527 *Dr. Hwang. But in seriousness, I think the first thing
2528 that we can do from the Congress's perspective is
2529 reauthorizing the user fee agreements and funding the NIH.

2530 And assuming that we have those things in place, I refer

2531 to some of the recommendations that I have in my written
2532 statement. Some of those include improving transparency so
2533 that sponsors know how to run trials and also learn from past
2534 failures. And the second is increasing the reliability of
2535 data that is imported and used to support new drug

2536 applications here. And part of that involves ensuring that
2537 data from non-U.S. trial sites are valid and that they are
2538 done with integrity.

2539 *Mr. Carter of Louisiana. How damaging is it to
2540 threaten NIH funding? And how -- what impact does that have
2541 on our budding researchers who are looking to study here in
2542 America and continue when they see the threat that NIH
2543 funding may be put on a political chopping block?

2544 *Dr. Hwang. I think it is incredibly damaging. All of
2545 the research that I know of points to the incredible work
2546 that the NIH does and the importance of it for new medicines,
2547 and I worry that the next generation of world-class talent
2548 will leave this country because of those cuts.

2549 *Mr. Carter of Louisiana. And we must do everything in
2550 our power to ensure that the best and brightest that is here
2551 stay here, the best and the brightest that want to come here
2552 to study to find cures for dreaded diseases continue to want
2553 to come here. And that is why we must maintain our
2554 leadership role and funding for NIH.

2555 My time is ended, I yield.

2556 *Mr. Griffith. The gentleman yields back. I now
2557 recognize the gentleman from New York, Mr. Langworthy.

2558 *Mr. Langworthy. Thank you, Mr. Chairman.

2559 The United States has long been the world's leader in
2560 biomedical innovation, and American researchers,
2561 universities, and life science companies have developed
2562 groundbreaking treatments that have improved in many cases,
2563 saved millions of lives of people not just here in the United
2564 States, but around the world. But we can't take that for
2565 granted.

2566 As other countries work aggressively to attract
2567 biomedical research in early-stage clinical development, we
2568 should be looking for opportunities to modernize outdated
2569 processes, embrace new technologies, and remove unnecessary
2570 barriers that slow motion.

2571 One area where I think we are seeing some of the most
2572 promising advances in the early stages of drug development --
2573 and with that, Dr. Verst, is you may know -- much of the pre-
2574 clinical drug development process still relies on laboratory
2575 and animal models for dosing and toxicity studies. As
2576 technologies like AI, virtual control groups, and other new
2577 approaches and methodologies continue to advance, where do
2578 you see the greatest opportunities to safely reduce the
2579 reliance on animal models?

2580 And from IQVIA's global perspective, what scientific or

2581 regulatory barriers are preventing a broader adoption of
2582 those technologies here in the United States?

2583 *Dr. Verst. Terrific questions. I think, by far, New
2584 Approach Methods, or NAM, is the needle-mover in terms of
2585 pre-clinical acceleration of timelines.

2586 I think, as the agency, the FDA, just recently in
2587 Operation Trialblazer identified, in fact, a new quantitative
2588 pharmacological modeling on this very front that helps us
2589 with dose optimization which is, as you know, very critical
2590 in animal testing is getting the right dose, the right safe
2591 dose. And I think those are the methodologies that will
2592 significantly enhance the efficiency and acceleration in the
2593 pre-clinical space.

2594 And I think it is just the continuation of the agency
2595 providing clarity and the actual guidance that will be
2596 extremely helpful, and especially biotech, biopharma sponsors
2597 that so need this very consistent and clear guidance as they
2598 develop their drugs.

2599 *Mr. Langworthy. I appreciate that, and I am encouraged
2600 by the progress we are seeing with the tools like the virtual
2601 control groups and other validated alternatives. But we
2602 shouldn't continue relying on outdated testing models that
2603 are unnecessary and lead to the unnecessary slaughtering and
2604 untimely deaths of dogs and cats in this country simply
2605 because that is the way it has always been done. And if

2606 these approaches can provide the FDA with reliable scientific
2607 evidence that it needs, I think we have a real opportunity to
2608 stop the process of testing on cats and dogs in this country,
2609 and I believe there is a mandate that is growing from the
2610 American people, as the curtain has been pulled back on this
2611 process, that they don't want to see this as the way we are
2612 approving our drugs in this country. And it is just common
2613 sense.

2614 But that is only one piece of the puzzle. We need to
2615 make sure that the United States remains the best place in
2616 the world to conduct early-stage research and develop
2617 medicines, because I don't -- I want to lead this country to
2618 ban animal testing on dogs and cats. I don't want to see it
2619 just all offshored to China, where they are going to do it
2620 without any humanity whatsoever.

2621 So Ms. Winckler, what best practices should FDA
2622 implement to make the United States the most attractive
2623 environment for early-stage research and clinical
2624 development?

2625 *Ms. Winckler. First and foremost, it is risk
2626 proportionate IND requirements. So saying for the risk of
2627 this product and for the -- you know, is it going into
2628 healthy volunteers or into patients? What do we need to see
2629 in that IND, and also then focusing FDA resources on the more
2630 novel interventions where we might have more safety

2631 questions.

2632 Another mechanism is continuing FDA's efforts in the new
2633 alternative methodologies that Dr. Verst mentioned. And in
2634 particular, not -- we shouldn't be looking for methodologies
2635 that replace what we learn -- what -- that replace the animal
2636 models; we should be looking for new methodologies that give
2637 us the information that we were looking for in the animal
2638 models. And those are two different questions.

2639 And so we should be exploring and sharing what were --
2640 what toxicity were we looking for, and how could we do that
2641 in a different way than using animal models.

2642 We also need to strengthen our phase one intensiveness,
2643 but primarily it is a risk proportionate approach to IND
2644 submission and transparency about that process and a rolling
2645 application.

2646 *Mr. Langworthy. Thank you very much. I could ask you
2647 questions all day, but I am out of time so I will submit some
2648 more for the record.

2649 [The information follows:]

2650

2651 *****COMMITTEE INSERT*****

2652

2653 *Mr. Langworthy. And I yield back, Mr. Chairman.

2654 *Mr. Griffith. The gentleman yields back. I now
2655 recognize Mr. Landsman of Ohio for his five minutes of
2656 questioning.

2657 *Mr. Landsman. Thank you, Mr. Chair, and thank you all

2658 for being here. I want to talk about biosimilars and get
2659 some feedback on how to get some of these really important
2660 drugs to market faster.

2661 So as you all know, these are generics. And, you know,
2662 in Europe, if it is approved, it is approved and we move
2663 forward -- or they move forward. And here, if it is approved
2664 there is additional testing and so on and so forth. And so
2665 it leads to -- you know, it is a very time-consuming, costly
2666 process. And patients end up paying more for prescription
2667 drugs.

2668 So one of the things that we have proposed,
2669 Representative Pfluger and I, is the Biosimilar Red Tape
2670 Elimination Act which would remove these extra steps and
2671 lower the cost for patients. Dr. Winckler, given -- Ms.
2672 Winckler, sorry, I promoted you. I am sorry.

2673 *Mr. Griffith. She has got a JD.

2674 *Mr. Landsman. She has got a JD, exactly.

2675 Ms. Winckler, you gave that sense, you know, it was --
2676 she has got to be a doctor. Given the FDA's current
2677 framework, what changes at the FDA would best support

2678 biosimilars and other generics entering the market? You are
2679 very close to this, obviously.

2680 *Ms. Winckler. Yes, so the Foundation facilitated a
2681 series of conversations with biosimilar developers who had
2682 not had much interaction with the FDA. And what we learned
2683 from that project was at some level not surprising. What the
2684 biosimilar developer said is we need more clarity about what
2685 is required, right? Like, what exactly do we have to do?

2686 And then the rationale for it, as well as, you know,
2687 just more conversations where the FDA may be -- have
2688 expertise in the project -- the product, rather -- where the
2689 company is trying to develop a biosimilar. How much of that
2690 can be shared?

2691 So it is clarity, it is continued learning and sharing
2692 the information, and then we should recognize that some of
2693 the challenges in the biosimilars market are actually not
2694 with FDA and getting them to the market, but their adoption
2695 and pull-through. So FDA can certainly do some --

2696 *Mr. Landsman. But is it not true that when the FDA
2697 approves a biosimilar there is still additional testing,
2698 whereas, say in Europe, if it is approved it is approved?

2699 *Ms. Winckler. Well, so it is approved there, and there
2700 are some continuing monitoring. But the main challenge that
2701 we have seen is that it tends to be payer adoption that
2702 presents some of these actually getting to patients.

2703 *Mr. Landsman. What does that mean? Sorry.

2704 *Ms. Winckler. In that if I am setting a formulary I
2705 may choose to continue to have one of those products, rather
2706 than having -- part of the reason the price drops when you
2707 have generics or biosimilars is when you have a number of
2708 them entering the market. In order to enter the market
2709 successfully you need to actually reach patients so that you
2710 have dollars returning. If the payment structure prohibits
2711 that getting to the patient and actually getting those
2712 dollars, then you don't have -- it -- you -- biosimilars
2713 won't enter the market.

2714 *Mr. Landsman. If you could change it tomorrow, today,
2715 is there any one big thing that would help expedite this that
2716 you think makes a lot of sense, or --

2717 *Ms. Winckler. I am always looking for the one big
2718 thing, and this doesn't have one. But there is -- I think
2719 FDA has made strides in being more clear about what is
2720 actually required, and then sharing that information with the
2721 biosimilar community. And --

2722 *Mr. Landsman. Our sense is that there is just too much
2723 red tape when it comes to these things. And so I guess the
2724 question -- I mean, you know, the clarity obviously helps,
2725 but I -- it also just seems to me that this is a -- this has
2726 already been tested, it has already been approved, it should
2727 just, you know --

2728 *Ms. Winckler. Yes. Well, and the structure for
2729 biosimilars is, you know, much newer than the structure for
2730 generic drugs, but it is not new. And so that -- it is an
2731 opportunity where we have learnings. And perhaps applying
2732 those learnings and changing the standards, it may be time.

2733 *Mr. Landsman. Dr. Kowalski, could you speak to the
2734 importance of bringing more of these biosimilars, generics to
2735 market for patients? Especially, you know, we talked about
2736 -- we have talked a lot about capping the cost of insulin for

2737 seniors and how this could increase competition and lower
2738 costs.

2739 *Dr. Kowalski. You know, so we strongly support the
2740 bipartisan INSULIN Act.

2741 Insulin, as I said in my opening statement, is mandatory
2742 for life with people with type 1 diabetes. And we have seen
2743 people rationing insulin and even dying for rationing of
2744 insulin. So any mechanism that will drive costs to be
2745 affordable and effective so people aren't choosing between
2746 rent or car payment and their insulin --

2747 *Mr. Landsman. I have cut you off, so I am sorry. I
2748 went over. I -- but I appreciate that and I agree. Thank
2749 you.

2750 I yield back.

2751 *Mr. Griffith. The gentleman yields back. I now
2752 recognize the gentlelady from Washington, Dr. Schrier, for

2753 her five minutes of questioning.

2754 *Ms. Schrier. Thank you, Mr. Chairman, and thank you
2755 for that answer about insulin. Anything we can do to bring
2756 down the cost of a medication that people like me would die
2757 without within days, I think -- I am not going to test it --
2758 is really important. So thank you to all of our witnesses.

2759 I wanted to talk first about biosecurity and research
2760 and global competition. Seventeen years ago the share of
2761 clinical trials started by Chinese companies was only one
2762 percent. Last year China-headquartered companies accounted
2763 for 39 percent of global oncology clinical trial starts and
2764 36 percent of vaccine trial starts, and that is massive
2765 growth and they are quintupling on their investment in
2766 research and development. There are really practical
2767 implications to this.

2768 I would just point to COVID as an example. The first
2769 Chinese-developed COVID vaccine showed significantly lower
2770 efficacy than the one in the U.S., ranging from 50.4 percent
2771 to about 60 percent. By comparison, the first American-made
2772 mRNA vaccines were about 95 percent effective, and American

2773 innovation and rigorous standards supported by a fully-
2774 staffed FDA saved millions of lives during the pandemic. I
2775 am thinking about the next pandemic. And if we yield our
2776 biomedical leadership to adversaries, the consequences for
2777 our country could be catastrophic, including whether our

2778 adversaries would even share those vaccines with us.

2779 Ms. Winckler, when early-stage cancer and vaccine trials
2780 migrate to China because the regulatory pipeline is faster
2781 and cheaper, what does that mean for the safety of Americans?

2782 And in a world where China, say, leads, for example, in
2783 drug research and development, how much longer would it take
2784 American patients to get those breakthrough medications and
2785 treatments?

2786 *Ms. Winckler. So part of the challenge here too is,
2787 right, when we have the innovation here we are learning here
2788 and we are having the local and better understanding of the
2789 product, and we can innovate more quickly. When that
2790 innovation is not here, we need to learn from it.

2791 And so the -- I think the challenge here is how do we
2792 better improve the U.S. system so that folks will want to be
2793 here because there is a risk of others -- there is the risk
2794 of what we do not control being weaponized against the United
2795 States, and particularly in biomedical innovation and simply
2796 access to biomedical products or food or, you know, these
2797 things that are essential to life. We should be positioning
2798 the U.S. for greatest success so that we are less susceptible
2799 to such challenges.

2800 *Ms. Schrier. I completely agree. All we have to do is
2801 reflect on where we were with regard to masks and gowns five
2802 years ago, and that really paints the picture of what it

2803 means when you get into more complicated and scientific
2804 elements.

2805 I wanted to pivot a little bit to vaccines because there
2806 has been a lot of discussion, information, and a lot of
2807 misinformation about vaccine safety. And I wanted to just
2808 point out first, long before a vaccine ever reaches a clinic
2809 the FDA does really thorough vetting and the determination of
2810 safety and efficacy through gold standard clinical trials.
2811 And once it is licensed, it then goes to the CDC's Advisory
2812 Committee on Immunization Practices, or ACIP, where they look
2813 at FDA data, pour over these thousands of pages, and then
2814 recommend exactly for whom and at what age and on what
2815 schedule a vaccine should be administered to best protect our
2816 country.

2817 The ACIP recommendation process has saved millions of
2818 lives, but its authority really depends on scientific
2819 credibility, and that is why Secretary Kennedy's overhaul of
2820 ACIP has been so concerning. When we inject or when he
2821 injected politics into a committee meant for objective
2822 medical decision-making, we threaten the hard-earned trust
2823 that parents and physicians like myself rely on every day.

2824 To any of the panelists who want to comment just in 25
2825 seconds here, in light of the recent political overhauls of
2826 advisory committees like ACIP, how can Congress ensure that
2827 FDA's clinical trial evaluations remain insulated from

2828 politics and stick with science?

2829 *Ms. Winckler. So I will just make one observation, and
2830 to remind us that when FDA approves a product, whether it is
2831 a vaccine or a drug or a biologic or a device, their job is
2832 not over. FDA conducts and monitors -- and product sponsors
2833 do, as well -- post-market surveillance so the learning
2834 continues. So I would just underscore that it is -- we
2835 sometimes think about the review and approval process as
2836 ending. It actually begins the next stage of scientific
2837 understanding which is continuing to gather information about
2838 how that product performs, and continuing to monitor for
2839 negative effects which is an important part of the entire
2840 medical product regulation system.

2841 *Ms. Schrier. Thank you. I want to double-click on
2842 that because there are some things that happen in one in two
2843 million people --

2844 *Mr. Griffith. And --

2845 *Ms. Schrier. -- and you don't know that until it is
2846 rolled out. Thank you for that.

2847 *Mr. Griffith. And the gentlelady yields back.

2848 *Ms. Schrier. I yield back.

2849 *Mr. Griffith. And I now recognize the gentlelady from
2850 Massachusetts, Mrs. Trahan.

2851 *Mrs. Trahan. Thank you, Mr. Chair. I am glad we are
2852 holding this hearing about biomedical innovation, which is

2853 absolutely critical in my home state of Massachusetts and to
2854 every American who is desperately waiting on a cure.

2855 This hearing is based on a real threat that we are
2856 losing our edge to China. But I want to be clear, you know,
2857 under this Administration we are not being outpaced. We are
2858 forfeiting. Before any promising new therapy even reaches
2859 the FDA there is discovery, there is pre-clinical work, there
2860 is first-in-human testing, and almost all of it traces back
2861 to NIH. Nearly every drug the FDA approves is linked to NIH-
2862 funded research. So talking about late-stage clinical trials
2863 when the entire pipeline has been under siege feels like we
2864 are rearranging the deck chairs on the Titanic.

2865 Dr. Verst, your testimony notes that emerging pre-
2866 commercial biopharma companies drive more than three-quarters
2867 of new phase one trial starts. Is it fair to say that these
2868 small biotechs are some of the most vital for the innovation
2869 pipeline?

2870 *Dr. Verst. Indeed, I think that these are critical
2871 customers -- or sponsors, rather -- in terms of fueling and
2872 promulgating our innovation, as you rightly highlight. In
2873 fact, the 75 percent being phase one in orientation in terms
2874 of the predominance of the biotech sector.

2875 But in addition, 65 percent of phase one through three
2876 clinical trials globally are actually conducted and invested
2877 by this biotech community.

2878 *Mrs. Trahan. And these are the most sensitive to
2879 capital constraints.

2880 Dr. Bollyky, can you elaborate on what happens when one
2881 of those small companies loses its funding in the early
2882 stages? Does that science wait patiently for them to
2883 recover? Does it go somewhere else or does it die?
2884 Enlighten us.

2885 *Mr. Bollyky. It -- science doesn't wait patiently. So
2886 small biotech firms in China are taking advantage of the
2887 opportunity to do regulatory arbitrage --

2888 *Mrs. Trahan. Yes.

2889 *Mr. Bollyky. -- by going through a pre-IND process of
2890 being able to conduct first-in-human trials much quicker, to
2891 be able to fast-follow the innovations that occur in the
2892 U.S., and to be able to seek licensing deals for that
2893 research.

2894 *Mrs. Trahan. The district I represent is home to
2895 companies just like that. I would like to share one of them.
2896 Versatope Therapeutics is a small biotech, just a couple
2897 dozen employees, that grew out of UMass Lowell's incubator.
2898 They were developing two critical innovations: a malaria
2899 vaccine and a universal flu vaccine. One shot, lifetime
2900 protection, both NIH-funded. But last year NIH terminated
2901 Versatope's flu vaccine grant for convenience. Their malaria
2902 grant renewal was frozen for months. And although it was

2903 ultimately reinstated, the damage had already been done. Ten
2904 scientists were furloughed. It is possible that the products
2905 they were working on will never see a pivotal clinical trial.

2906 And as if that wasn't bad enough, it gets worse. OMB
2907 just proposed a rule that takes that exact mechanism,
2908 termination for convenience, and makes it the law for every
2909 Federal grant in our country. That same rule replaces the
2910 scientific review process with political appointees, upending
2911 years of stability that has made the United States the global
2912 gold standard when it comes to biomedical research.

2913 Dr. Kowalski, Breakthrough T1D submitted comments on the
2914 rule earlier this week. Can you explain what it means for
2915 early-stage researchers to have this codified in Federal
2916 regulation?

2917 And how will it change what recourse a company has when
2918 its grant is terminated?

2919 *Dr. Kowalski. Well, Breakthrough T1D did sign on to a
2920 United for Cures Coalition letter to the -- both the House
2921 and the Senate requesting that proposal be withdrawn. We
2922 feel very strongly that letting political appointees override
2923 scientific peer review puts decisions, real breakthrough
2924 therapies, at risk --

2925 *Mrs. Trahan. Right.

2926 *Dr. Kowalski. -- as you have described.

2927 And having grants terminated for convenience threatens

2928 long-time studies and significant progress made -- making --
2929 that we are making towards breakthrough therapies.

2930 *Mrs. Trahan. I agree. You know, that is why I led
2931 more than 100 Members warning OMB about the potentially

2932 disastrous consequence on this rule, specifically on our
2933 biomedical research infrastructure.

2934 I would like to enter into the record the response we
2935 received from Director Vought.

2936 *Mr. Griffith. And the date?

2937 *Mrs. Trahan. It is dated June 30.

2938 *Mr. Griffith. Okay. Of this year?

2939 *Mrs. Trahan. Yes.

2940 *Mr. Griffith. All right. Without objection --

2941 *Mrs. Trahan. It doesn't seem to me like --

2942 *Mr. Griffith. Without objection, so ordered.

2943 [The information follows:]

2944

2945 *****COMMITTEE INSERT*****

2946

2947 *Mrs. Trahan. Thank you. It doesn't seem to me like
2948 they are taking full and fair consideration of the almost
2949 350,000 public comments they have received. In fact, this

2950 response doubles down on the rule calling NIH grants too
2951 woke, and recycling old conspiracy theories. It is just
2952 deeply unserious.

2953 This Administration says America should be the best
2954 place in the world to develop medicines, and all of us here
2955 agree with that. But you can't be that place while signing
2956 innovative companies' death warrants and calling it
2957 convenience.

2958 Thank you, Mr. Chairman. I yield back.

2959 *Mr. Griffith. And Mrs. Trahan, if you will, make sure
2960 you get a copy of that so we can have it for the record.

2961 I now recognize the gentleman from Texas, Mr. Veasey,
2962 for his five minutes of questioning.

2963 *Mr. Veasey. Thank you, Mr. Chairman. I know that much
2964 of today's discussion has focused on how Congress can
2965 modernize clinical trials and make them faster and more
2966 efficient. And I think those goals are very important,
2967 particularly as advances in science and technology create new
2968 opportunities to bring innovative therapies to patients more
2969 quickly.

2970 At the same time -- I think especially we learned a lot
2971 of this lesson around COVID-19 -- making sure that we ensure

2972 that clinical research reflects the diversity of the
2973 population that we have here and who is ultimately going to
2974 use these therapies is going to be very critical to
2975 generating evidence that is both scientifically robust and
2976 applicable.

2977 Dr. Verst, given your experience designing and
2978 implementing these clinical trials across a wide array, how
2979 can Congress ensure that efforts to modernize clinical trials
2980 also expand participation amongst historically under-
2981 represented communities?

2982 Again, particularly when you take into consideration a
2983 lot of the noise that had -- we had to go through during
2984 COVID-19 to get people to just to take that vaccine.

2985 *Dr. Verst. It is a terrific question, and certainly,
2986 you know, a challenge on the clinical trial front.

2987 I think that, first and foremost, understanding how to
2988 recruit and incentivize community physicians to participate
2989 in clinical trials. And of course, it is incumbent upon us
2990 as an industry to ensure that we reduce the burden in
2991 participation.

2992 And beginning as -- Dr. Winckler, I give you an honorary
2993 degree --

2994 *Ms. Winckler. Thank you.

2995 *Dr. Verst. -- mentioned in terms of -- the academic
2996 medical centers are critically important, but so are the hub-

2997 and-spoke referral methodologies. If we are able to put the
2998 right technology systems in place within healthcare systems,
2999 EMR technology that can be interoperable with electronic data
3000 capture associated with clinical trials, one would then be
3001 able to identify the right patient at the right time on their
3002 clinical journey to be eligible for certain clinical trials.

3003 That is just one example of how to access those
3004 community physicians and, of course, their respective patient
3005 population to help encourage more participation in clinical
3006 trials. And we call that clinical research really as a
3007 treatment option.

3008 *Mr. Veasey. Yes, yes. Wow, wow. That is there.

3009 I would also like to touch on the FDA's approval process
3010 which has long relied on evidence demonstrating that new
3011 therapies are both safe and effective. And as advances in
3012 science create opportunities to use novel endpoints and
3013 biomarkers in clinical development, we are considering how
3014 these tools can complement traditional approaches. And I
3015 know that this -- the challenge is ensuring that greater
3016 flexibility strengthens the clinical research enterprise
3017 while preserving the high evidentiary standards that
3018 providers rely on.

3019 And Dr. Kowalski, your testimony supports modernizing
3020 clinical requirements including increased use of novel
3021 endpoints and biomarkers. Dr. Kowalski, what safeguards are

3022 going to be needed to ensure that these new approaches
3023 continue to generate reliable evidence and maintain public
3024 confidence?

3025 *Dr. Kowalski. Sure. I think having a robust FDA that
3026 is staffed appropriately and following clear and consistent
3027 regulatory expectations will help us lead with safety and
3028 then follow with efficacy.

3029 I think in our patient community we have seen a -- the
3030 pace not meet the sense of urgency. And that -- again, the
3031 opportunity cost as you are living with a dangerous disease
3032 every day where you are dosing a life-threatening drug in our
3033 patient community.

3034 *Mr. Veasey. Yes.

3035 *Dr. Kowalski. So I think that risk benefit analysis
3036 has to be clear, and then we need the right staff to then
3037 follow up on executing against the regulations.

3038 Our community is desperate for innovation, and we are
3039 seeing it and we are on the cusp of it. And I think if we
3040 lead with safety, you will see innovative therapies reaching
3041 the population and driving benefit.

3042 *Mr. Veasey. Yes. No, absolutely. Thank you.

3043 Mr. Chairman, I yield back. Thank you.

3044 *Mr. Griffith. The gentleman has yielded back and we
3045 have Mr. Auchincloss on the way. I would ask the witnesses,
3046 do you all have time to wait a minute or so? Now, if he is

3047 20 minutes away, we won't wait. But if he shows up in the
3048 next minute or two, we will hold if you all are okay with
3049 that.

3050 *Dr. Kowalski. That is great.

3051 *Mr. Griffith. All right, thank you.

3052 [Pause.]

3053 *Mr. Griffith. All right, we had turned the clock on,
3054 we were going to give you another five minutes. You are down
3055 -- you still had 4 minutes and 30 seconds, but I am glad you
3056 didn't wait -- you didn't push us to the test.

3057 I now recognize the gentleman from Massachusetts, Mr.
3058 Auchincloss, for his five minutes of questioning.

3059 *Mr. Auchincloss. Thank you, Chairman, and thank you in
3060 particular for your ongoing partnership as we work to
3061 modernize clinical trials in the United States with
3062 bipartisan legislation currently in discussion draft form
3063 which we have gotten substantive feedback on, and hopefully
3064 soon in legislative form. I want to discuss that with the
3065 witnesses here, and I have appreciated the written testimony.

3066 My first question is for you, Ms. Winckler. We have
3067 seen many areas of overlap between the research that Reagan-
3068 Udall has done on clinical trials modernization; Operation
3069 Trialblazer from the Administration; and then our own
3070 legislative draft including risk-based, non-clinical testing,
3071 patient-centric patient matching; increased FDA communication

3072 and transparency; shared learning to the agency's application
3073 of flexible evidentiary strategies and adaptive trial
3074 designs; and better use of existing data such as natural
3075 history studies and real-world evidence.

3076 What are key considerations for Congress to support FDA
3077 consistency and transparency in trial design, accepted
3078 endpoints, and natural history studies while maintaining a
3079 flexible regulatory environment?

3080 *Ms. Winckler. So key is part of the -- what you just
3081 talked about, where it is a consistent message from what
3082 Congress is creating and what stakeholders are saying and
3083 what HHS is saying. That, seeing the same threads in a
3084 common direction, is incredibly helpful to the FDA staff. It
3085 is saying, yes, this is how we pursue it.

3086 And then continuing with that is the clear question of
3087 is there clarity in the authorizing statute. Then the agency
3088 has the opportunity to provide the detail and regulations and
3089 the resources and staff to implement it.

3090 *Mr. Auchincloss. Part of it Congress can legislate,
3091 and we should. And our bill really focuses on the things
3092 that only Congress can do, particularly creating a point-of-
3093 care clinical trial enrollment process because only, you
3094 know, maybe five to seven percent of eligible U.S. patients
3095 are enrolled at any given time. And if we want to widen the
3096 throughput of U.S. clinical trials, we just have to make it

3097 easier to recruit and enroll, right? And I think Congress
3098 has a special role there.

3099 A lot of it can be done by FDA leadership, though, in
3100 terms of that trial design, accepted endpoints, consistency.
3101 FDA has had a hurly burly last 18 months, and now has an
3102 opportunity for a reset after regrettably hemorrhaging a lot
3103 of competence and credibility under the leadership -- the
3104 previous leadership. What advice would you give to an
3105 incoming FDA commissioner about how to restore a culture of
3106 high standards for safety and efficacy, and also the kind of
3107 consistency and early engagement that creates certainty for
3108 investors and investigators?

3109 *Ms. Winckler. So obviously, that job is an incredibly
3110 challenging one. But the way to approach it is thinking
3111 about the stability for the staff. So what is it that the
3112 agency is pursuing? What are the priorities? And what are
3113 the expectations of them? And that is that they discharge
3114 their work as strong, competent scientists and lawyers and
3115 all of the other staff who are experts in the law and the
3116 regulatory structure.

3117 And in order to do their job well, they have to interact
3118 with the experts outside the FDA to help with the emerging
3119 science and the emerging products. So underscoring that
3120 stability within the agency as well as the -- really, the
3121 essential communication and interaction with external

3122 stakeholders in order to keep pace with the science -- to be
3123 a science-based regulator you have to keep pace with the
3124 science.

3125 *Mr. Auchincloss. Which, you know, of course, is what
3126 Reagan-Udall has been designed to do, right?

3127 And I do think for that -- for those FDA regulators,
3128 that human -- one of the things I hear from investigators all
3129 the time is the importance of that human-to-human interaction
3130 early on in the process, just sit across from the table with
3131 each other and just go through, point by point, what are we
3132 looking for for endpoints? What are we looking for for trial
3133 design? My sense has been that that highly iterative
3134 interactive process has degraded over time. And not just --
3135 I wouldn't say not just under the previous leader, but maybe
3136 really over the last 5 to 10 years. And I think the next FDA
3137 leadership has to just inculcate that culture again of, you
3138 know, really engage deeply with your investigator.

3139 *Ms. Winckler. Exactly. Some, I think wrongly, call
3140 interaction between the regulator and regulated industry as
3141 industry capture. I think to be a functional regulator you
3142 have to interact with the industry that you are regulating so
3143 that you understand their challenges.

3144 *Mr. Auchincloss. Specifically early on.

3145 *Ms. Winckler. The regulator still makes the decision.

3146 *Mr. Auchincloss. Yes, specifically early on.

3147 Well, much more but I will yield back to the chairman.

3148 *Mr. Griffith. I thank the gentleman and now recognize
3149 the gentleman from California, Mr. Mullin, for his five
3150 minutes of questioning.

3151 *Mr. Mullin. Thank you, Mr. Chair, and thank you to our
3152 witnesses for being here today. I am glad we have convened
3153 this important hearing to discuss the U.S. leadership and the
3154 FDA's role in drug development.

3155 I am very proud to represent California's 15th
3156 congressional district, otherwise known as the birthplace of
3157 biotechnology. Lifesaving therapies and cures are researched
3158 and developed every day in my hometown of south San
3159 Francisco, pushing the boundaries of what we thought was
3160 scientifically possible and expanding our ability to keep our
3161 loved ones healthy.

3162 Before discussing ways the FDA can advance drug
3163 development and the review process, we must first address the
3164 unforced errors coming from this Administration that is
3165 ultimately slowing down drug development and potentially
3166 preventing the next groundbreaking therapy from making it to
3167 patients. Attacks on the NIH, research cuts, and recent OMB
3168 efforts to politicize independent scientific research are all
3169 pushing innovators to study and work abroad. We are going to
3170 lose a generation of the leading scientific minds to
3171 President Trump, and the majority continue to undermine

3172 American innovation.

3173 On top of that, preventable upheaval at the FDA is
3174 hurting domestic drug development. I recently sent a letter
3175 to the FDA outlining how the agency's staffing cuts,
3176 leadership turnover, and inconsistent priorities is adding
3177 uncertainty to the system and making it increasingly
3178 difficult for manufacturers to work with the FDA. And those

3179 ultimately hurt by the FDA's delayed communications and
3180 changing guidance will be patients who must go even longer
3181 without the therapy that could change their life for the
3182 better.

3183 So Dr. Kowalski, thank you for being here. From the
3184 patient advocate perspective, could you please outline what
3185 the impacts would be if the next therapy or cure in
3186 development for type 1 diabetes, for example, was delayed due
3187 to preventable factors?

3188 *Dr. Kowalski. I mean, I think the answer is quite
3189 obvious. It would be devastating.

3190 I mean, our patient community, we have seen amazing
3191 advancements in my lifetime. My brother started on urine
3192 glucose testing, and here we are now wearing automated
3193 insulin delivery systems, thanks to NIH support, partnership
3194 with FDA, partnership with companies. And now here we are on
3195 the cusp of curing the disease and preventing the disease.
3196 And to lose that would just be absolutely devastating.

3197 *Mr. Mullin. Thank you for that. We cannot lose the
3198 momentum in so many ways.

3199 And in addition to pushing back on ways this
3200 Administration has slowed U.S. drug development, we must also
3201 be forward-thinking about ways for the FDA to modernize
3202 processes as the science continues to advance. So Ms.
3203 Winckler, your testimony highlighted that current FDA
3204 communication with drug sponsors leads to guesswork and
3205 overly conservative assumptions that ultimately slows down
3206 the process. So what more does the FDA and Congress need to
3207 do to streamline these interactions and ensure responses are
3208 clear and prompt?

3209 *Ms. Winckler. Part of it is simply having the
3210 opportunity for more interaction, particularly early-stage,
3211 smaller companies to be able to have that conversation. And
3212 then the -- there are risk proportionate requirements. So
3213 what is the risk of the IND and are you having phase
3214 appropriate requirements for that?

3215 And then it is -- the clarity and speed of communication
3216 is essential. And then we have to tackle the other part too,
3217 where sometimes sponsors assume because someone else has done
3218 something they must do it as well. So they -- the data
3219 packages get bigger and bigger because they saw someone else
3220 do it. We have to clarify that, in fact, that is not the
3221 approach that may be needed.

3222 *Mr. Mullin. Thank you for that.

3223 So let me just conclude by saying it is vital that we
3224 meet the scientific and medical needs today by continuing to
3225 modernize and streamline FDA's drug review process. I look
3226 forward to continuing this work to get therapies to patients
3227 faster. Thank you all for being here.

3228 Thank you, Mr. Chair, for allowing me to waive on, and
3229 with that I will yield back.

3230 *Mr. Griffith. The gentleman yields back. That brings
3231 us to the end. I would like to thank all of our witnesses
3232 again for being here.

3233 I was accusing Ms. DeGette of having more people in the
3234 back, like a clown car, and bringing more out.

3235 [Laughter.]

3236 *Mr. Griffith. But she told me she only had five more,
3237 and apparently they didn't make it before I closed.

3238 I will remind members -- excuse me. Thank you all for
3239 being here. Members will have additional questions for you
3240 all subsequent to the hearing. I will remind members they
3241 have 10 business days to submit their questions for the
3242 record, and I ask the witnesses to respond to the questions
3243 promptly. Members should submit their questions by the close
3244 of business on Wednesday, July 29.

3245 And we -- let's see, I must have missed a page. We
3246 approve all the other items that were on the documents for

3247 the -- there we go.

3248 I ask unanimous consent to insert into the record the
3249 documents included on the staff hearings document list, along
3250 with the other ones that we accepted during the hearing.

3251 And without objection, so ordered.

3252 [The information follows:]

3253

3254 *****COMMITTEE INSERT*****

3255

3256 *Mr. Griffith. That being said, and without objection,
3257 the subcommittee is adjourned.

3258 [Whereupon, at 1:19 p.m., the subcommittee was
3259 adjourned.]