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1 | ANDERSON COURT REPORTING
2 | RPTS WATSON
3 | HIF203.140
4 | 21ST CENTURY CURES: EXAMINING BARRIERS TO ONGOING EVIDENCE
5 | DEVELOPMENT AND COMMUNICATION
6 | TUESDAY, JULY 22, 2014
7 | House of Representatives
8 | Committee on Energy and Commerce,
9 | Subcommittee on Health
10 | Washington, D.C.

11 | The Subcommittee met, pursuant to call, at 3:00 p.m., in
12 | Room 2322, Rayburn House Office Building. Hon. Joseph R.
13 | Pitts [chairman of the subcommittee] presiding.

14 | Present: Representatives Pitts, Burgess, Shimkus,
15 | Blackburn, Lance, Bilirakis, Ellmers, Pallone, Green, Barrow,
16 | DeGette, and Waxman (*ex officio*).

17 | Staff: Leighton Brown, Press Assistant; Noelle Clemente,
18 | Press Secretary; Sydne Harwick, Legislative Clerk; Robert
19 | Horne, Professional Staff Member, Health; Carly McWilliams,
20 | Professional Staff Member, Health; Chris Sarley, Policy

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21 | Coordinator, Environment & Economy; Heidi Stirrup, Health
22 | Policy Coordinator; Jessica Wilkerson, Legislative Clerk;
23 | Ziky Ababiya, Staff Assistant; Eric Flamm, FDA Detailee;
24 | Eddie Garcia, Professional Staff Member; Karen Nelson, Deputy
25 | Committee Staff Director for Health.

26

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27 Mr. PITTS. The subcommittee will come to order. The
28 chair will recognize himself for an opening statement.

29 In this the sixth hearing of our 21st Century Cures
30 Initiative, we are examining continued evidence development
31 and communication of information regarding treatments and
32 cures in the real world setting. Discovery of the risks and
33 benefits of drug or treatment does not end with FDA approval
34 or clearance. It is often just the beginning of learning
35 about different drugs and devices, for different indications,
36 conditions, and populations. Treatment in the real world
37 also brings out additional information on safety and
38 efficacy, and ensuring that this knowledge is shared widely
39 among providers, patients, and researchers is critical.

40 As a result, the ability of patients, physicians, and
41 developers to communicate effectively is so important for the
42 future of cures in this country. Unfortunately, many of the
43 witnesses and participants we have had before us since the
44 Cures Initiative began have raised concerns regarding
45 barriers to communication and evidence development. This
46 hearing is a direct result of the feedback we have received
47 from patient groups and other interested parties.

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48 As today's witnesses will discuss efforts to limit off
49 label use among the provider community, limitations on
50 communication found under HIPAA, and the Physician's Sunshine
51 Act are just a few of the barriers to 21st century cures that
52 have been raised with us over the past few months. It is my
53 hope that this hearing allows the members an opportunity to
54 consider those potential barriers and the role they play in
55 our healthcare system.

56 With that thought in mind, I would like to thank all of
57 our witnesses for being here today, and I will yield the
58 balance of my time to Dr. Burgess, vice chairman of the
59 subcommittee.

60 [The information follows:]

61

62 ***** COMMITTEE INSERT *****

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64 Dr. BURGESS. Thank you, Mr. Chairman. I, too, want to
65 welcome our witnesses. Certainly look forward to hearing
66 from them today.

67 I appreciate the continued series of hearings on the
68 21st Century Cures Initiatives. Certainly looking forward
69 today to exploring the role that healthcare providers,
70 physicians, can have in increasing communications between
71 patients, researchers, and those who innovate. Different
72 uses for therapies are constantly being discovered through
73 information highways, including social networks, patient
74 advocacy groups, and physicians sharing information.

75 There is no doubt that technology and the ability to
76 communicate easily with people all around the world will
77 change how we conduct research, how clinical trials are
78 managed, and how the post-market works.

79 We must recognize this fact and be open to rethinking
80 the traditional means of how we have engaged with our
81 patients. We must also rethink about expectations of the
82 ease with which patients may engage with each other. The
83 fact of the matter is if I get on a plane with my iPad, I
84 have got the *New England Journal*, I have got the *Journal of*

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85 | *the American Medical Association*, and I have got the most
86 | current *Journal of Obstetrics and Gynecology* with me. And it
87 | is simply a matter of opening it and reading while on the
88 | plane. The ability to keep up with rapidly-changing and
89 | evolving fields is unlike anything anyone has ever had in the
90 | past.

91 | So this is the world in which we live today, and we need
92 | to open to realizing the benefits that can be drawn from this
93 | fact. And also recognize that while we are exchanging
94 | information, patient advocacy groups are likewise engaged.

95 | So we certainly look forward to a lively discussion with
96 | the panel today. Mr. Chairman, I will yield back the time.

97 | [The information follows:]

98 |

99 | ***** COMMITTEE INSERT *****

100 |

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101 Mr. PITTS. Anyone on our side seek time? Vice chair,
102 Ms. Blackburn?

103 Mrs. BLACKBURN. Thank you so much, Mr. Chairman. And
104 to our panel, I want to welcome each of you. There is so
105 much that is going on in the field of healthcare informatics,
106 and Dr. Burgess just touched a little bit on that, and also
107 medical devices. We are going to hear from Edwards Life
108 Sciences about a heart valve which was approved in 41
109 countries before it was approved here in the U.S.

110 And this is something that is unacceptable when you look
111 at the length of time that it takes to get these medical
112 devices through the FDA's process. In Memphis, Tennessee, my
113 home State, one in four jobs is dependent on medical devices.
114 And when you look at what is happening in the Nashville area
115 with healthcare, healthcare informatics, you realize the
116 importance and the increasing importance of that as an
117 economic development sector to our State.

118 I think it is imperative that we provide a 21st century
119 regulatory framework for 21st century technology and a
120 framework that is going to encourage innovation while
121 providing safe, effective, and new therapies. And with that,

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122 | I yield back my time.

123 | [The information follows:]

124 |

125 | ***** COMMITTEE INSERT *****

126 |

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127 Mr. PITTS. The chair thanks the gentlelady. Now
128 filling in for the ranking member, Mr. Pallone, Mr. Green of
129 Texas, 5 minutes for opening statement.

130 Mr. GREEN. Thank you, Mr. Chairman. And thank you and
131 the ranking member, who will be here shortly, on this
132 continuing series of hearings on the 21st Century Cures.
133 This is really what our Health Subcommittee should be about,
134 how we can help. And following my colleague my Tennessee,
135 although I did not know that many jobs in Memphis were for
136 medical. I thought it was just barbecue or Graceland.

137 Mrs. BLACKBURN. If the gentleman will yield --

138 Mr. GREEN. Briefly.

139 Mrs. BLACKBURN. -- it is because of the barbecue that
140 we need the medical --

141 Mr. GREEN. Well, as you know, there is a difference
142 between Tennessee and Texas barbecue. We like --

143 Mrs. BLACKBURN. I would ask the gentleman to yield
144 again on that. There would not be a Texas if there were not
145 Tennessee --

146 Mr. GREEN. Well, and I cannot disagree with that
147 because, frankly, we got all the rebels from Tennessee and

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148 | helped us win independence in Texas. But with that, I am
149 | going to yield the balance of my time to my colleague,
150 | Congressman DeGette from Colorado.

151 | Ms. DeGETTE. Thank goodness. Mr. Chairman, I really
152 | want to thank you for holding this next hearing in this
153 | series on the 21st Century Cures. I have got to say I was
154 | around my district all weekend, and everybody I talked to
155 | from the Jefferson County Economic Development Team to the
156 | telephone town hall meeting I had last night, to the OFA
157 | people. Everybody was excited to hear about this bipartisan
158 | effort that we are having, and I am excited, too.

159 | Throughout all of the previous hearings and roundtables
160 | that we have had on all of these topics, we have already
161 | learned a tremendous amount about what role Congress should
162 | play in helping to further advance and accelerate treatment
163 | and cures.

164 | Today the witnesses will talk about examining barriers
165 | to ongoing evidence development and communication. The
166 | potential areas for discussion are far ranging, to say the
167 | least, but I am looking forward to hearing some specifics
168 | from the witnesses on the potential benefits of enhanced data

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169 collection and improved maintenance and secured sharing of
170 data and information.

171 These types of evidence development and communication
172 can and do play essential roles in the drug and device
173 development and approval processes, as well as in
174 reimbursement determinations. For example, how can we take
175 advantage of data and information to more effectively
176 identify patients for clinical trials that are relevant to
177 their individual disease or condition? How can we harness
178 the data and information collected during clinical trials?
179 What about information after the drug or device is introduced
180 into the market? And how do we effectively utilize this
181 information while maintaining a high standard of privacy
182 protections?

183 On the reimbursement side, how is Medicare's coverage
184 with the evidence development process currently being used?
185 And how can we improve these processes to be clear?

186 Just to talk for a minute about some of the things that
187 are going on in terms of evidence sharing and data, Mr.
188 Burgess talked about taking his iPad on the airplane. And I
189 just literally got off the airplane from Denver where I was

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190 reading this article from *The New Yorker* this week. Maybe
191 some of you have seen it. It is about a family who has a
192 child with a very, very, very, very rare genetic disorder,
193 NGLY1. And they finally got it diagnosed, they did not think
194 anybody else had it until the dad, who is a computer
195 professor at the University of Utah, wrote a blog which went
196 viral, and everybody read about it.

197 And the upshot is that they have now identified patients
198 with this genetic disorder around the world. They have all
199 met. They have put together a research consortium. They
200 have people doing research and writing a paper to be
201 published in a scientific journal. And they are on their way
202 to try to figure out what they can do about this very, very
203 rare defect.

204 These patients did this on their own because they were
205 sophisticated parents. So what I would like to know is what
206 can we do to harness this in a much more systemic way so that
207 these types of communications can occur effortlessly both
208 within the United States and with our colleagues around the
209 world. So all of these are important questions.

210 I really look forward to hearing the testimony today and

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211 | to learning about these topics. Thank you very much, and I

212 | yield back.

213 | [The information follows:]

214 |

215 | ***** COMMITTEE INSERT *****

216 |

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217 Mr. PITTS. The chair thanks the gentlelady, and now
218 recognizes the ranking member of the full committee, Mr.
219 Waxman, 5 minutes for opening statement.

220 Mr. WAXMAN. Thank you very much, Mr. Chairman. Today
221 we have an opportunity to learn more about several issues
222 that were raised at our previous meetings on the 21st Century
223 Cures Initiative. From the first roundtable discussion that
224 kicked off the initiative, we heard that FDA and NIH are
225 leaders in driving and using advances in molecular medicine
226 and digital technology to help get new cures to patients more
227 quickly. They have also made great strides in improving and
228 streamlining procedures for conducting clinical trials and in
229 reviewing innovative new drugs and medical devices.

230 However, we also heard about impediments that stand in
231 the way of researchers and companies making full use of these
232 advances. While patient registries can facilitate enrollment
233 in clinical trials and help researchers find new research
234 avenues to pursue, many believe more could be done to
235 encourage their development and use.

236 Electronic health care records can help physicians and
237 sponsors identify patients for clinical trials and evaluate

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238 | the effects of drugs already on the market, but privacy
239 | concerns are limiting their use. And although FDA has shown
240 | an increasing willingness to accept data from smaller
241 | clinical trials, the more limited data generated to support
242 | FDA approval may not be adequate for coverage decisions by
243 | Medicare or private insurers. I look forward to hearing more
244 | about these barriers and what can be done to address them.

245 | We should remember, though, that we have a review and
246 | approval system that is already working quite well. It has
247 | led to enormous breakthroughs and coverage of cutting-edge
248 | drugs and devices. FDA reviews and approves drugs faster
249 | than any other regulatory agency in the world. NIH and FDA
250 | are world leaders in clinical trial design and in integrating
251 | the newest science into their policies and approaches while
252 | protecting the health of the patients. And Medicare has
253 | demonstrated flexibility in its national coverage
254 | determinations so that beneficiaries can access these new
255 | cures.

256 | I have a great interest in fostering greater access to
257 | innovative drugs, devices, and health services. But I also
258 | know that access to new, innovative medicine alone will not

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259 | increase the quality and outcomes patients experience in our
260 | healthcare system. Incentives must be in place for providers
261 | to furnish high quality care to the right patient at the
262 | right time in the right setting of care.

263 | The Affordable Care Act was a major advancement in
264 | meeting these challenges, but we still have work to do. In
265 | particular, we should enact the delivery reforms contained in
266 | our bipartisan SGR legislation. We can make another great
267 | stride forward if we can send this legislation to the
268 | President's desk before the end of this year.

269 | I have a little time left, and I would be pleased -- oh,
270 | anybody on our side want it?

271 | If not, I yield back the time, and let us hear from the
272 | witnesses.

273 | [The information follows:]

274 |

275 | ***** COMMITTEE INSERT *****

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277 Mr. PITTS. The chair thanks the gentleman. As always,
278 members' opening written statements will be made a part of
279 the record.

280 We have one panel today with five witnesses. I will
281 introduce them in the order of them making their
282 presentations. First, Dr. Josh Rising, director of medical
283 devices, the Pew Charitable Trust; Dr. Louis Jacques, senior
284 vice president, chief clinical officer of ADVI; Mr. Michael
285 Mussallem, chairman and chief executive officer of Edwards
286 Life Sciences Corporation; Dr. Gregory Schimizzi, co-founder,
287 Carolina Arthritis Associates, P.A.; and Ms. Mary Grealy,
288 president, Healthcare Leadership Council.

289 Thank you each for coming. Your written testimony will
290 be placed in the record. You will each be given 5 minutes to
291 summarize your testimony. And at this time we will recognize
292 Dr. Rising, 5 minutes, for his opening statement.

293

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294 | STATEMENTS OF JOSH RISING, DIRECTOR, MEDICAL DEVICES, THE PEW
295 | CHARITABLE TRUSTS; LOUIS JACQUES, SENIOR VICE PRESIDENT AND
296 | CHIEF CLINICAL OFFICER, ADVI; MICHAEL A. MUSSALLEM, CHAIRMAN
297 | AND CEO, EDWARDS LIFESCIENCES; GREGORY SCHIMIZZI, CO-FOUNDER,
298 | CAROLINA ARTHRITIS ASSOCIATES; MARY GREALY, PRESIDENT,
299 | HEALTHCARE LEADERSHIP COUNCIL

300

301

302 | STATEMENT OF JOSH RISING

303

304 | Dr. RISING. Chairman Pitts, Ranking Member Pallone,
305 | members of the committee, I thank you for the opportunity to
306 | provide testimony. My name is Josh Rising. I am a physician
307 | director of medical devices at the Pew Charitable Trusts.

308 | We have an exciting opportunity today to talk about the
309 | future of healthcare, a future where we can harness
310 | electronic data to improve patient care. Advances in
311 | technology offer a potential for new approaches to develop
312 | medical evidence through a continuous cycle that begins
313 | before a product is approved and continues as the product is
314 | used by patients.

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315 As we move towards this total life cycle approach, we
316 must consider two important issues. First, we know that
317 clinical trials are the largest contributor to the cost and
318 length of product development. We need to use new approaches
319 to decrease their length and cost without doing away with
320 these trials and the critical data they provide. Second, we
321 must have the tools necessary to quickly and efficiently
322 identify problems with approved drugs and medical devices,
323 and to assess their performance in real world settings that
324 can be different from clinical trials.

325 We are at a key turning point. Electronic health
326 records today collect more data on patient outcomes than we
327 have ever had, but we are failing to realize that potential.
328 One important innovation to harness data from electronic
329 health records is the registry, large databases that collect
330 information on groups of patients treated for a particular
331 medical condition.

332 Now, imagine if we could conduct clinical trials for a
333 tenth of the current cost. This is precisely what physicians
334 in Sweden recently did using an existing registry. They
335 studied heart attack prevention in more than 7,000 patients,

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336 comparing two different procedures. The data were drawn from
337 electronic health records, and the trial cost only \$300,000,
338 or roughly \$50 per patient. Conducting such a study outside
339 of a registry in the United States would cost hundreds of
340 millions of dollars, if not more. We can do this in the
341 United States, too, but only if we fix the lack of
342 interoperability among electronic health records and
343 streamline certain electronic administrative processes.

344 Second, just as important as ensuring prompt access to
345 new cures is the ability to detect problems with drugs and
346 medical devices on the market and assess their performance in
347 real world conditions. Here, too, registries can help. For
348 example, an Australian registry of artificial joints found
349 that one type of Metal-on-Metal Hip failed at a rate more
350 than two times higher than conventional hips, ultimately
351 leading to a worldwide recall of the device. Detecting such
352 problems earlier is vital for patient safety and could save
353 our healthcare system vast sums.

354 Pew will soon release a report on registries produced in
355 partnership with the Blue Cross and Blue Shield Association
356 and the Medical Device Safety Group and the EPINet. In this

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357 | report, we recommend steps to deliver timely, actionable
358 | information from registries to all stakeholders, including
359 | the public.

360 | Now, there are other ways that electronic data can also
361 | improve patient care. One is better use of the brand new
362 | Unique Device Identifier, or UDI, System, which was created
363 | by FDA at the direction of Congress and will result in a
364 | unique number assigned to nearly all medical devices. If we
365 | now incorporate this number into insurance claims, we can use
366 | FDA's Sentinel System to assess device safety problems the
367 | same way we do for drugs. Incorporating UDI into claims will
368 | also provide payers, such as CMS, with the necessary data
369 | unavailable elsewhere, to evaluate outcomes for patients with
370 | implanted medical devices.

371 | Adding a UDI field to claims has generated support
372 | across healthcare, including from hospitals, such as
373 | Geisinger and Mercy, health plans like Aetna, physician
374 | societies, including the American College of Cardiology and
375 | the Society of Thoracic Surgeons, as well as patient and
376 | consumer organizations. Additionally, HHS Secretary Burwell
377 | articulated the benefits of adding UDI to claims during her

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378 | Senate confirmation process.

379 | New mechanisms to collect data both prior to and after
380 | FDA approval can help facilitate faster clinical trials and
381 | ensure that any problems are promptly detected. Congress
382 | should work with the Administration to maximize the potential
383 | of these new data sources to ensure patient access to safe
384 | and effective medical devices.

385 | Thank you again for the opportunity to testify, and I
386 | welcome your questions.

387 | [The prepared statement of Dr. Rising follows:]

388 |

389 | ***** INSERT 1 *****

390 |

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391 | Mr. PITTS. The chair thanks the gentleman, and now
392 | recognizes Dr. Jacques 5 minutes for an opening statement.
393 |

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394 STATEMENT OF LOUIS JACQUES

395

396 Mr. JACQUES. Chairman Pitts, Ranking Member Pallone,
397 and members of the subcommittee, my name is Louis Jacques.
398 From October 2009 through February 2014 I was the director of
399 the Coverage and Analysis Group at the Centers for Medicare
400 and Medicaid Services. I was the division director in that
401 group from June 2004 until 2009.

402 We implemented coverage with evidence development and
403 the FDA/CMS Parallel Review Pilot Initiative. We also
404 revised CMS regulations pertaining to Medicaid coverage and
405 FDA-approved investigational device exemption clinical
406 trials, and executed a memorandum of understanding between
407 FDA and CMA.

408 CMS experience over the past decade is illustrative of
409 the challenges to the wide adoption of certain innovative
410 technologies. One, there are innovative products and
411 services that do not clearly fall within the statutory scope
412 of the Medicaid program benefit. Two, the available evidence
413 at the time of initial marketing may not clearly establish
414 the clinical value of a new technology in the relevant

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415 beneficiary population. Three, historic coding paradigms can
416 be uninformative to the extent that the insurer cannot
417 identify the specific item or service for which it is paying.
418 This blind buying creates reluctance among insurers and
419 hampers the establishment of brand value for high performing
420 technologies.

421 I believe there are opportunities. External
422 stakeholders have requested more opportunities for coverage
423 with evidence development and FDA/CMS parallel review. While
424 these programs were articulated in the early 2000s by a prior
425 Administration, both are included in the 2012 White House
426 National Bio Economy Blueprint.

427 Since 2009, CED has essentially replaced non-coverage in
428 final national coverage determinations, thereby furnishing
429 Medicaid coverage when it would otherwise have not been
430 available. By contrast, in the 5 years before 2009, almost
431 half of all national coverage determinations ended with non-
432 coverage.

433 Unfortunately, CMS' ability to furnish CED is limited.
434 CMS initiates CED under ARC's statutory authority. CMS
435 implements CED through the formal national coverage

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436 | determination process. Due largely to staffing cuts the
437 | annual number of NCDs published has dropped from
438 | approximately 12 to 13 in fiscal years 2007 and 2008 to only
439 | five in 2012 and six in 2013. Current staffing is
440 | approximately half of 2007 levels.

441 | Under parallel review, both FDA and CMS maintain their
442 | separate standards. I have no reason to believe that either
443 | agency has toughened its process as a process of parallel
444 | review. While the structure of the pilot contemplates the
445 | possibility of a national coverage determination, parallel
446 | review does not inherently require that CMS undertake the NCD
447 | process. The content of the parallel review engagement
448 | depends on the product's development stage. Ideally, early
449 | discussions with CMS could result in more persuasive pivotal
450 | trial, evidence that leads local Medicare contractors to
451 | uniform coverage.

452 | Results to date are encouraging. One product received
453 | unanimous yes votes and positive comments at its recent FDA
454 | panel meeting, which the company credited to the discussions
455 | with both agencies that inform the design of the pivotal
456 | trial. CMS does not have sufficient staff to match FDA's

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457 | bandwidth on potential parallel review candidates. Despite
458 | interest from device manufacturers, the parallel review pilot
459 | has been limited to only two products.

460 | In conclusion, CMS review of clinical trials serves
461 | three goals. First, it provides important financial support
462 | for innovation. Second, the sponsor can obtain CMS feedback
463 | on whether the initial trial design could persuasively inform
464 | a coverage decision. Third, CMS can inform the sponsor of
465 | existing coding or payment paradigms that may apply to the
466 | product.

467 | The current vehicles for coverage in clinical trials are
468 | unnecessarily siloed, preventing the publication of an
469 | integrated, comprehensive policy. I believe this could be
470 | fixed with small changes in state. The definition of a local
471 | coverage determination could be revised to align LCD
472 | authority with the actual scope of local contractor claims
473 | processing responsibility, thereby enhancing transparency and
474 | predictability. As an alternative to non-coverage, some
475 | stakeholders have expressed interest in new payment paradigms
476 | for early stage devices with immature evidence bases.

477 | Acknowledging the challenges of the Federal

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478 administration budget, stable funding sources should be
479 considered for these initiatives that are expected to produce
480 downstream benefits. Their investment requires funding that
481 is more predictable potentially from the Medicare Trust Fund
482 itself.

483 Thank you for the opportunity to share my thoughts, and
484 I would be happy to answer any questions.

485 [The prepared statement of Mr. Jacques follows:]

486

487 ***** INSERT 2 *****

488

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489

Mr. PITTS. The chair thanks the gentleman.

490

Mr. Mussallem, you are recognized for 5 minutes.

491

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492 STATEMENT OF MICHAEL A. MUSSALLEM

493

494 Mr. MUSSALLEM. Yes. Thank you very much, Mr. Chairman
495 Pitts, Ranking Member Pallone, Congresswoman DeGette, and
496 members of the subcommittee. My name is Mike Mussallem. I
497 am the chairman and CEO of Edwards Life Sciences. I am truly
498 honored to join the other panelists today to discuss the path
499 to revitalizing medical device innovation in the United
500 States.

501 I and the other employees of Edwards Life Sciences, from
502 our engineers to our valve assemblers, share a passion for
503 helping patients. I am privileged to lead a company that is
504 the world leader, and has been for 50 years, in heart valve
505 replacements.

506 The reason I am here is that I am worried about
507 innovation in the U.S. and that it is suffering from
508 increasingly costly, and cumbersome, and a risk averse
509 culture in our regulatory and payment systems. Our recent
510 experience with a transformative therapy to heart valve
511 replacement patients gives us a unique perspective on the
512 current climate.

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513 In short, Edwards Technology allows a heart team to
514 deliver a collapsible prosthetic valve through a catheter
515 into the body to avoid cracking the chest, stopping the
516 heart, and avoid the long and painful recovery that goes
517 along with that open heart surgery.

518 This has become the most extensively studied heart valve
519 ever, including an unprecedented four *New England Journal of*
520 *Medicine* articles that demonstrated a triple win, which is a
521 substantial and sustainable clinical effect, cost
522 effectiveness, and extraordinary quality of life improvement.

523 We appreciated a productive relationship with Dr. Jeff
524 Shuren in FDA, as well as Dr. Patrick Conway and his
525 colleagues at CMS, whose approach ensured that there was a
526 balanced and reasonable process for this transformative
527 therapy.

528 Also in a remarkable effort of groundbreaking
529 collaboration between medical societies, regulators, and
530 other stakeholders, we built a comprehensive clinical
531 evidence and quality measurement tool for this therapy called
532 the TBT registry.

533 But there is room for improvement. We all know the path

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534 | to approval and reimbursement is not easy, and it should not
535 | be. Yet the U.S. approval of this American technology
536 | trailed Europe by 4 years. We are pleased that the FDA
537 | leadership viewed this delay as a catalyst to improve, and we
538 | see several opportunities to remove barriers. I am going to
539 | focus on three.

540 | Number one, evidence development mechanisms can be
541 | improved to reduce cost and delay. FDA had recently proposed
542 | a number of improvements to the pre-market clinical trial
543 | process and the post-market surveillance process that hold
544 | the promise. And these have been discussed at this
545 | committee. In my view, when registries are done right, they
546 | can yield extremely useful information about patients'
547 | outcome and device benefits.

548 | However, the clinical and scientific benefits of
549 | registries must be balanced with a potentially significant
550 | cost burden, complexity, and potential misuse of that data.
551 | In our case, many physicians told us it takes longer to fill
552 | out the 300 fields in the TBT registry than it does to
553 | perform the procedure itself.

554 | Number two, reimbursement incentives need to be aligned

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555 | with promoting innovation. Efforts to curb healthcare
556 | spending could have the unintentional consequence of slowing
557 | down innovation in our cost-cutting frenzy. It is imperative
558 | to recognize that medical device innovations become more
559 | effective and more efficient with time and with experience.
560 | We need a system that does not shut the door to reimbursement
561 | on day one.

562 | In select cases, coverage with evidence development can
563 | be a tool that allows promising technologies to reach
564 | patients sooner while developing evidence to support lasting
565 | reimbursement. And finally, FDA's vision to improve the
566 | regulatory process must be accelerated. Dr. Shuren and his
567 | team have outlined strategic priorities that strike the right
568 | balance between pre-market and post-market data collection
569 | and improving customer service.

570 | We know FDA is a complex bureaucracy to manage, and our
571 | leaders need a mandate to change more quickly. Congress
572 | could encourage FDA by providing additional support to
573 | expedite these changes and give them room to innovate.

574 | And finally, no discussion about medical technology is
575 | complete without understanding the true impact that they have

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576 | on patients. And we meet a lot of patients. To mention one,
577 | Lester Tenney, a true American hero, part of our Greatest
578 | Generation, survivor of the Bataan Death March, and a Japan
579 | POW, had long sought an apology from the Japanese government
580 | on behalf of Federal soldiers. Unfortunately, just as this
581 | apology was agreed upon, he was diagnosed with disabling and
582 | inoperable aortic stenosis. He would not survive long, let
583 | alone long enough to make this trip.

584 | The good news is that Lester received an Edwards trans-
585 | catheter heart valve, was able to travel to Japan, get the
586 | apology. This would not have been possible even 5 years
587 | earlier. And he remains vital to this day and dedicated to
588 | helping veterans. Lester and tens of thousands of other
589 | patients we have had the opportunity remind us every day that
590 | our work is personal. It impacts people individually.

591 | Thank you for the opportunity to testify today.

592 | [The prepared statement of Mr. Mussallem follows:]

593 |

594 | ***** INSERT 3 *****

595 |

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596 | Mr. PITTS. The chair thanks the gentleman. |

597 | Dr. Schimizzi, you are recognized for 5 minutes for an
598 | opening statement. |

599

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600 STATEMENT OF GREGORY A. SCHIMIZZI

601

602 Dr. SCHIMIZZI. Chairman Pitts, Ranking Member Pallone,
603 members of the subcommittee, and honored guests, it is a
604 distinct honor to be here today and testify before you. My
605 name is Gregory Schimizzi, and I am testifying before you as
606 a member of the board of directors and past president of the
607 Coalition of State Rheumatology Organizations, or CSRO. And
608 I am a private practice rheumatologist at the Carolina
609 Arthritis Associates in Wilmington, North Carolina.

610 The CSRO appreciates the opportunity to share our views
611 related to barriers to ongoing evidence development and
612 communication transparency. Specifically, I will focus on
613 situations in which valid communication pathways are being
614 hampered by outdated practices of the Food and Drug
615 Administration, or FDA, as well as touch upon some unintended
616 consequence of the Sunshine Act, or open payments, as
617 implemented by the Centers for Medicare and Medicaid
618 Services, or CMS.

619 The FDA does not allow pharmaceutical companies to
620 actively distribute key clinical information even if it is

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621 related to the on-label indicated, unless it is explicitly
622 referenced in the package insert of that product. By
623 limiting the sharing of information, physicians are hampered
624 in their ability to gain all of the firm scientific rationale
625 and medical evidence needed to treat patients.

626 So that clinicians may be better informed, the CSRO
627 urges the FDA to develop standards for qualifying real world
628 data through a public process, to expand the current process
629 of review of materials beyond what is included in the package
630 insert, to also cover other key data, such as sub-population,
631 pharmaco-economic, or comparative cost data, and to ensure a
632 timely review process for such information.

633 As part of the Affordable Care Act, Congress required
634 the Administration to set up a process by which transfers of
635 value from certain covered entities, primarily manufacturers
636 of drugs and devices to physicians, would be reportable.
637 Such reportable information would then be made publicly
638 available. The overall goal of this transparency is to make
639 particular potential financial conflicts of interest more
640 transparent.

641 However, there are considerable problems with the

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642 current implementation of open payments, including the lack
643 of guidance and clarity regarding the physician registration
644 process, as well as the review of dispute process lacking
645 necessary protection for physicians.

646 Finally, a recent CMS-proposed rule related to open
647 payments would severely hamper the flow of information.
648 Therefore, the CSRO respectfully requests that CMS provide
649 additional provider-specific guidance for the registration
650 process and adopt policies that allow for flexibility of
651 enrollment requirements so that physicians struggling to
652 enroll remain able to participate in a meaningful manner,
653 ensure an impartial process for disputing the accuracy of
654 financial information intended for public disclosure, take
655 steps to enhance the fairness and accuracy of the program by
656 ensuring that healthcare providers have access to meaningful
657 mechanism for limiting the distribution of disputed
658 information, and reconsider its proposal to eliminate the
659 continuing medical education exemption, and instead
660 appropriately expand the list of certified CME accrediting or
661 issuing agencies beyond the five currently cited in
662 regulation.

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663 As I hope I have outlined today, current practices at
664 both the FDA and CMS may be inappropriately hampering the
665 exchange of information, making it difficult for physicians
666 to receive the information they need to make valuable
667 treatment decisions.

668 For the FDA, I hope that Congress will examine ways to
669 allow for more proactive changes among clinicians with
670 appropriate safeguards to ensure that such information is
671 truthful and not misleading. For CMS, I hope that Congress
672 can urge strategic plan programmatic changes to make the
673 transparency process accurate and appropriately descriptive
674 of the financial relationships among the various entities.

675 Thank you once again for allowing me to speak today and
676 to consider my comments today as well as the other
677 information captured in my written statement. The Coalition
678 of State Rheumatology Organizations looks forward to working
679 with the committee to address these issues. I look forward
680 to your questions. Thank you very much.

681 [The prepared statement of Dr. Schimizzi follows:]

682

683 ***** INSERT 4 *****

684

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685 | Mr. PITTS. The chair thanks the gentleman. |

686 | And now, Ms. Grealy, you are recognized for 5 minutes

687 | for an opening statement. |

688

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689 STATEMENT OF MARY GREALY

690

691 Ms. GREALY. Mr. Chairman and members of the
692 subcommittee, thank you for the opportunity to testify this
693 afternoon. And thank you as well for the attention you're
694 bringing to the future of American healthcare, and the
695 ability of the healthcare system to develop, communicate, and
696 utilize the data that can lead to 21st century cures.

697 I am here today representing the Healthcare Leadership
698 Council, a coalition of leaders from all sectors of American
699 healthcare. I am very proud that our membership includes
700 innovators, like Mr. Mussallem, also on today's witness
701 panel.

702 Our members share this committee's goals for a
703 healthcare system that is affordable, sustainable, and of the
704 highest attainable quality, and that is also on path toward
705 curing the diseases and illnesses that have cost us far too
706 much both in lives and resources.

707 Each year, those involved in all aspects of healthcare
708 generate literally trillions of decisions, communications,
709 interventions, consultations, treatments, therapies, and

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710 | clinical trials. The key to achieving progress lies in
711 | harnessing this massive amount of information and setting
712 | policies and practices in place to productively share and to
713 | use this data.

714 | HLC members have been engaged in this challenge for some
715 | time both as individual innovative companies and
716 | collectively. What I share with you today is our broad-
717 | based, multi-sector perspective on how we can create an
718 | environment in which data can be used to strengthen the
719 | entirety of the healthcare continuum.

720 | There are three areas where I will focus my comments
721 | today. One, the role of the HIPAA privacy law; two, the need
722 | for Federal data policies that enhance access to information
723 | to enable health system improvements and accelerated medical
724 | research; and three, the potential impact of the new Sunshine
725 | Act on the physician industry collaborations that are
726 | critical engines of healthcare advancement.

727 | On the first point, the HIPAA privacy and security laws
728 | are generally serving patients in the healthcare system well,
729 | and should continue to be the guiding rule regarding the
730 | appropriate and effective use of patient health data. There

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731 are certain aspects of HIPAA, however, that warrant
732 continuing review and discussion.

733 We need to keep in mind that HIPAA was created at a time
734 in which policymakers were not thinking about the knowledge
735 that could be gained by accessing data residing in large
736 databases and the technological ability to process that data
737 very rapidly. It may be necessary to adjust the
738 authorization components of HIPAA to ensure that data can be
739 used effectively for research.

740 Also, in order to transmit data and collaborate in its
741 use, we need to review the utility of having 50 separate sets
742 of State privacy laws and regulations instead of a single
743 national standard.

744 On the issue of Federal data policy, Healthcare
745 Leadership Council members have developed a set of consensus
746 multi-sector principles on data policy that I have submitted
747 for the record. One of these key principles is our belief
748 that access to Federal health data should no longer be denied
749 to entities perceived to have a commercial interest. The
750 profit status of an organization should not take precedence
751 over the larger question of how best to conquer disease and

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752 | improve population health.

753 | Any standard that restricts access to critical,
754 | federally-held health data is, in fact, detrimental to our
755 | shared goals for medical and human progress. We must put the
756 | benefit to patients first.

757 | Finally, we believe strongly that Congress must
758 | diligently monitor the impact of the Physician Payment
759 | Sunshine Act. This is not a criticism of transparency, which
760 | our member companies practice and HLC strongly endorses. We
761 | are concerned, though, about the transparency without
762 | context. We are concerned that physicians may feel
763 | stigmatized by the Federal reporting of their interactions
764 | with manufacturers in a way that does not communicate the
765 | patient benefits of such collaborations.

766 | Some of our member companies are already witnessing
767 | physicians withdrawing from collaborative activities, which
768 | can have a devastating impact not only innovation, but also
769 | on product efficacy and safety. Congress should monitor the
770 | implementation of this law to ensure that both transparency
771 | and innovation are fully achieved.

772 | Mr. Chairman, thank you again for the opportunity to

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773 | testify today. We believe that this committee's bipartisan
774 | vision for 21st century cures is an achievable reality, one
775 | that can be accelerated by creating a pathway for the
776 | productive use of data that we already possess.

777 | Thank you, and I will be happy to answer any questions.

778 | [The prepared statement of Ms. Grealy follows:]

779 |

780 | ***** INSERT 5 *****

781 |

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782 Mr. PITTS. The chair thanks the gentlelady. Thanks to
783 all the witnesses for their testimony. I will begin
784 questioning and recognize myself for 5 minutes for that
785 purpose. I will start with you, Dr. Schimizzi.

786 Different uses for FDA-approved drugs and devices are
787 constantly being discovered, many times for treatment of
788 different conditions and diseases or for different
789 populations. Manufacturers of these products have access to
790 robust data sets and information that is not always limited
791 to the specific indications listed in their package inserts.

792 Why is it important that we responsibly allow providers
793 to have access to such information to ensure that the most
794 appropriate treatment options are being considered?

795 Dr. SCHIMIZZI. Well, thank you, Mr. Chairman. In
796 rheumatology we see many patients with rare diseases and
797 unusual autoimmune problems. And we also see patients who
798 are referred to us by other specialists for autoimmune
799 problems in their specialty that they do not know how to
800 handle, so they send them to us.

801 In our armamentarium of medications, we have an array of
802 medications that work very well. Some of them are of low

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803 toxicity, and some of them are of high toxicity. In the
804 event of a new agent being brought to the United States
805 medical arena having a high safety profile, but lack an
806 indication for an orphan disease or a critically important
807 problem in another organ system, like the eye, for example,
808 use of those medications would be miraculous and have a high
809 margin of safety if we had access to information. I am just
810 using the eye as an example. There are other instances as
811 well. Primary muscle disease is another one.

812 Medications are available, but the indications are not
813 there, and they probably never will be because the numbers of
814 patients who have these diseases is so small, it would take
815 many years to discover that the indications were there and
816 millions of dollars, perhaps tens of millions of dollars, to
817 identify that.

818 So if we had access to information that these new
819 medications might be effective in certain other small
820 diseases that may have been gleaned from the data that was
821 derived from the direct clinical trials, then that would be
822 extremely helpful to us and help guide us in a direction that
823 would increase efficacy, and increase safety, and maybe even

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824 decrease cost and poor outcomes.

825 Mr. PITTS. Do you believe that the current restrictions
826 on off label communications are limiting healthcare
827 professionals' ability to provide the most appropriate
828 treatment to patients? And if so, what needs to happen?

829 Dr. SCHIMIZZI. What was the last part?

830 Mr. PITTS. If so, what needs to happen?

831 Dr. SCHIMIZZI. Yes, I do believe that the limitation of
832 exchange of information is hampering the delivery of
833 healthcare to some of these patients, especially in my sub-
834 specialty. What needs to happen is that we need to have
835 access to information that is locked up in vaults in
836 pharmaceutical companies, locked up in data sets in study
837 information.

838 For instance, here is a great example that I can spread
839 to rheumatology. There is a great drug that came out many
840 years ago to prevent ulcers and to treat ulcer disease and
841 esophagitis called Prilosec. The generic name was
842 omeprazole. Prilosec was a mixture of two different mirror
843 image molecules, D-enantiomer and an L-enantiomer. It is
844 like a right hand and a left hand.

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845 Well, it came to light that one of the enantiomers was
846 much more effective at treating ulcer disease and
847 esophagitis, so out came esomeprazole, or Nexium, which has
848 proven to be more effective.

849 What if there were medications on the market right now
850 that we have that could treat diseases but have side effects,
851 and yet if we isolated the D-enantiomer and the L-enantiomer,
852 we would identify which one was effective and which one
853 caused the problems. I submit to you that there are drugs in
854 our compendium right now that have D and L isomers, and the
855 data sets are probably available in the vaults of
856 pharmaceutical manufacturers that show the D isomer is more
857 effective than the L isomer. The L isomer has more
858 complications than the D isomer. So that would be a dramatic
859 improvement.

860 So such data sets are locked up. We do not have access
861 to them, and I do not know that we ever will.

862 Mr. PITTS. Quickly, Ms. Grealy, you mentioned HIPAA.
863 What kind of changes should we consider to HIPAA to ensure
864 that big data can be used effectively for research purposes
865 while still protecting patient privacy?

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866 Ms. GREALY. Well, when the HIPAA law was originally
867 passed, there was an exemption for healthcare operations, and
868 that included the treatment and payment for patients. But
869 sort of outside that scope was research activity.

870 So I think a very simple approach would be let us
871 include healthcare research as if it is a natural part of
872 healthcare operations.

873 There are probably several other recommendations that we
874 could make, but I think the key here is to make sure that we
875 have an appropriate balance between protecting patient
876 information, and we believe very strongly in that. We also
877 do not want to erect barriers to having access to that data.

878 I think a big part of this is having informed consumers,
879 educated patients, and especially as we are seeing patients
880 engage more and more in the management of their own
881 healthcare.

882 Mr. PITTS. The chair thanks the gentlelady, and my time
883 has expired. The chair recognizes the ranking members, Mr.
884 Pallone, 5 minutes for questions.

885 Mr. PALLONE. I wanted to start with Dr. Jacques, and
886 then if I have time, ask Dr. Rising a question. But, Dr.

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887 | Jacques, I wanted to better understand what you mean when you
888 | talk about the confusion created by Medicare's vague
889 | authority and lack of administrative agility in Medicare
890 | coverage and payment policies for new innovation
891 | technologies.

892 | Could you briefly describe the statutory limitations
893 | that apply to Medicare coverage determinations, both as they
894 | relate to coverage with evidence development and local
895 | coverage determinations? And what are your recommendations
896 | for how to streamline these authorizes? And then maybe how
897 | does this existing authority impact the decision making
898 | framework for CED study questions, and what data is needed to
899 | trigger and end the CED study?

900 | Mr. JACQUES. The reasonable and necessary standard,
901 | which is essentially the coverage standard for Medicaid,
902 | those provisions are 1862(a)(1) of the Social Security Act,
903 | which is then followed by subsections (a) through (p) that
904 | parce things out for prevention hospice and things along
905 | those lines.

906 | CED itself is not defined in the Social Security Act, so
907 | CMS has had to rely on the Agency for Healthcare Research and

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908 | Qualities Research Authority under Section 1862(a)(1)(E) of
909 | the Act. Thus ARC has to approve every CED decision.

910 | While the scope of a national coverage determination is
911 | described broadly in statute as a decision under Title 18,
912 | local coverage determinations are defined in the Act only as
913 | decisions under 1862(a)(1)(A). Thus, an LCD could not
914 | implement coverage with evidence development. So even if
915 | there were an item or service that is only furnished within
916 | one contractor region of the entire country, a national
917 | decision would be required to implement CED.

918 | I have been told by various stakeholders that CED could
919 | be approached more eagerly if it were not tied to the formal
920 | NCD process. The current framework forces CMS to apply the
921 | CED requirement to all beneficiaries receiving the item or
922 | service in question, regardless of where they live. This is
923 | a particular challenge for beneficiaries who live in the
924 | remote parts of the country where clinical studies do not
925 | normally happen and clinical trial enrollment is, frankly,
926 | unrealistic for many.

927 | A more agile CED paradigm would permit CED to occur in
928 | parallel with other forms of coverage rather than requiring

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929 | everyone to fit through the same door.

930 | Mr. PALLONE. Now, you also highlight a rapid decline in
931 | the number of national coverage determinations in the last
932 | few years. How has the lack of staff resources within CMS
933 | impacted that decline, and what, if any, impact has this had
934 | on coverage with evidence development?

935 | Mr. JACQUES. I believe that staff reductions are the
936 | largest single cause of the decline in number of national
937 | coverage determinations. And the impact of this decline is
938 | broader than CED because it impacts the Agency's ability to
939 | respond to other requests for coverage.

940 | CED itself generally requires more internal staff work
941 | to develop, and it creates an ongoing need after the
942 | publication of the final decision to interact with sponsors
943 | who might want to conduct clinical trials. By my own
944 | estimate, it takes about three times as much internal effort
945 | for CMS staff to do CED than it does to simply say yes or no.

946 | Mr. PALLONE. All right. Thank you. And I am going to
947 | try to get this in. Dr. Rising, you note in your testimony
948 | that the taste trial conducted in Europe on heart attacks
949 | cost a tiny fraction, perhaps one-one hundredth of what it

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950 would cost in the U.S., because it was able to make use of
951 patient registries. However, we also heard in Mr.
952 Mussallem's written testimony that registries can be very
953 expensive to set up and maintain, and threshold questions
954 must be answered to determine when and how registries should
955 be used for post-market data collection.

956 Now, I am familiar with registries from the law creating
957 the 9/11 Health Program. It included a provision to
958 authorize a registry of people who were exposed to toxic dust
959 from the attack on the World Trade Center on 9/11. But I do
960 not know much about registries for assessing medical
961 products.

962 Can you explain how these registries work, and can you
963 describe what source of impediments to the use exist,
964 including why they may be harder, expensive, to set up and
965 maintain. And I would like to know your thoughts and what
966 can be done to facilitate their use.

967 Dr. RISING. Sure.

968 Mr. PALLONE. You do not have a lot of time to do it.

969 Dr. RISING. I will in 30 seconds.

970 Mr. PALLONE. Okay.

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971 Dr. RISING. So medical product registries, kind of like
972 the 9/11 responder registry, will follow a group of patients
973 with one exposure for a period of time. So, for example, we
974 heard a little bit from Mr. Mussallem about their trans-
975 catheter valve registry, which follows patients who have
976 gotten a valve for a period of time in order to assess their
977 long-term outcomes.

978 Now, while registries can be a tremendous source of
979 information on the post-market performance of devices, there
980 are some barriers to setting them up. And one of the biggest
981 barriers is the lack of interoperability between systems.
982 So, for example, clinical staff need to enter data in the TBT
983 registry and then enter a lot of the same data again in
984 electronic health records. So this kind of added burden on
985 the staff is one of the biggest drivers for why registries
986 are currently inefficient in the United States.

987 Now, in addition to these post-market benefits,
988 registries can have tremendous benefits for innovation as
989 well. One of the other benefits that we have seen for the
990 trans-catheter valve registry that Dr. Shuren talked about at
991 the first hearing was that data from the registry was used to

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992 expand an indication for the device.

993 So if we are able to take some steps in this country
994 forward for registries, we should be able to see significant
995 benefits both on the safety side and then also on the
996 innovation side of things.

997 Mr. PALLONE. All right. Thanks a lot. Thank you, Mr.
998 Chairman.

999 Mr. PITTS. The chair thanks the gentleman, and now
1000 recognizes the vice chairman of the full committee, Ms.
1001 Blackburn, 5 minutes for questions.

1002 Mrs. BLACKBURN. Thank you, Mr. Chairman. I have got
1003 just a couple of questions that I want to go to, and again, I
1004 thank you all for participating with us and working with us.

1005 There is a lot of bipartisan agreement on this. You
1006 have heard different members of the committee speak to that,
1007 finding a pathway forward so that we deal with the regulatory
1008 framework, provide some certainty, and speed up the process
1009 by which innovation and cures are going to get to our
1010 patients is a shared goal. And so, we thank you for that.

1011 Mr. Mussallem, let us go back to the topic of the
1012 registry. We have talked a little bit about that, and you

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1013 | have all talked about basically the data and the value of the
1014 | data that can be found within those registries and the
1015 | benefit to our -- to patients from being able to utilize the
1016 | data in those registries.

1017 | So let us talk a little bit about risks that are there
1018 | for the patients or cost that is there. And can you give me
1019 | just a little bit of an articulation looking at the other
1020 | side of this with risk and cost, both the actual dollar cost,
1021 | or, as Ms. Grealy mentioned, the privacy, some of the privacy
1022 | concerns?

1023 | Mr. MUSSALLEM. Yes, thank you very much, Member
1024 | Blackburn. And I applaud the bipartisan cooperation toward
1025 | this shared goal. Registries can be a powerful tool, and we
1026 | by and large think they could be very appropriately used. My
1027 | only caution that I mentioned in my testimony is that there
1028 | are some cases where technologies and/or therapies are well
1029 | enough known that can establish a safety and effectiveness
1030 | standard without going through that sort of process.

1031 | In the case of the TBT registry, in particular, that I
1032 | mentioned, the large group of stakeholders ended up
1033 | generating this long list of items to be collected. I

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1034 mentioned 300 data fields. Maybe when a technology is brand
1035 new and unknown you want to learn an awful lot about it. The
1036 problem is that becomes quite costly. And at some point, it
1037 gets too expensive to maintain.

1038 Ideally, you would have a registry that could be
1039 whittled down to those things that are really most critical
1040 that you would like to measure, and there may be a way to,
1041 you know, to populate it with electronic data that is already
1042 being generated, such that a registry could be a very cost-
1043 effective tool.

1044 In the case of the TBT registry, it literally cost seven
1045 figures plus per year for that total cost. That is shared by
1046 a lot of constituents, including manufacturers. But a lot of
1047 the burden rests on hospitals. They have a burden where they
1048 actually pay a fee every year and additionally have to put on
1049 dedicated staff just to fill out those fields. So not
1050 something to be taken lightly.

1051 Mrs. BLACKBURN. If you had to give us a list of guiding
1052 principles as we look at a framework for developing some of
1053 the registries, what are those three or four principles that
1054 you would articulate?

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1055 Mr. MUSSALLEM. You know, I think it is most important
1056 to have a clear risk benefit analysis and also have clear
1057 goals set out by the registry. There should be a set of
1058 rules around the registry and some governance guidelines
1059 around it.

1060 Dr. Rising spoke to the work that Pew Foundation has
1061 done in this area. It is actually very thoughtful, done with
1062 a broad group of stakeholders about the value of registries.
1063 And I think that is not a bad guidepost.

1064 Mrs. BLACKBURN. Okay. And let me ask you this. Do you
1065 envision any of these registries moving to the point where
1066 the patient could populate some of those cells and fields
1067 themselves?

1068 Mr. MUSSALLEM. You know, ideally registries would not
1069 be expensive to populate, and any time that they could be
1070 filled out automatically in an electronic patient record or
1071 even, as you suggest, that a patient could do it themselves,
1072 this is important. I mean, some simple things. Is the
1073 patient alive? Is the patient going through a routine of
1074 exercise, or what is the patient's diet? All these things
1075 are very potentially powerful variables that could provide

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1076 | insight to the value of technologies.

1077 | Mrs. BLACKBURN. Well, we would hope that anybody
1078 | populating one of these with an app on an iPad would indeed
1079 | be alive and not have their avatar doing it for them.

1080 | Mr. MUSSALLEM. Well said.

1081 | Mrs. BLACKBURN. So, okay. Dr. Schimizzi, let me ask
1082 | you just a couple of things on off label use. You had
1083 | mentioned that, and I am intrigued by this. I think this is
1084 | an area that it holds some promise. It is a legal practice,
1085 | correct?

1086 | Dr. SCHIMIZZI. Yes.

1087 | Mrs. BLACKBURN. Okay. Do you consider it a best
1088 | practice to inform a patient that a therapy that is being
1089 | prescribed is off label?

1090 | Dr. SCHIMIZZI. I think that is best practice, yes, and
1091 | I always do.

1092 | Mrs. BLACKBURN. If informed doctors can legally
1093 | prescribe off labeled patients who are also well informed,
1094 | what would be the current barriers to that practice?

1095 | Dr. SCHIMIZZI. Well, the barrier is we need the
1096 | information to pick which drug to use in a difficult

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1097 | situation. And that information is not always available to
1098 | us.

1099 | In the immune system, there are different cells that are
1100 | work, we know that a certain cell is active in one disease.
1101 | And if you suppress that cell, we can suppress the disease or
1102 | cure the disease. And that agent might be available for a
1103 | cancer, but if we can transpose and use that in this patient,
1104 | that would probably work. It would be very nice to have that
1105 | information from the pharmaceutical company or manufacturer
1106 | or innovator who developed that product that, yes, this is
1107 | very, very likely an effective way to use this medicine, but
1108 | we are never going to study it because they probably never
1109 | will.

1110 | Mrs. BLACKBURN. Okay. I will yield back. I am over
1111 | time. Thank you, Mr. Chairman.

1112 | Mr. PITTS. The chair thanks the gentlelady, and now
1113 | recognizes the ranking member of the full committee, Mr.
1114 | Waxman, 5 minutes of questions.

1115 | Mr. WAXMAN. Thank you, Mr. Chairman. The Affordable
1116 | Care Act strengthened our movement away from a healthcare
1117 | system that rewards providers for the volume of services they

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1118 provide and toward a system that fosters and promotes the
1119 high quality, high value care. The bipartisan SGR
1120 legislation as to make this committee's perspective a
1121 permanent change in the reimbursement rate for physicians
1122 under Medicare. That legislation passed out of this
1123 committee and the other committees' jurisdiction and then
1124 furthered that aim by incentivizing care delivery that is
1125 coordinated in alignment with consensus guidelines and best
1126 practices, and as efficient as it is appropriate.

1127 Dr. Jacques, in your testimony you speak of the broad
1128 national goals of Federal health agencies to improve public
1129 health and protect beneficiaries' access to products and
1130 services that demonstrate genuine benefit. You suggest that
1131 FDA approval for drugs and devices puts products on the
1132 shelves, but a prudent purchaser should not blindly pay for
1133 those products without regard to how useful or appropriate
1134 they are.

1135 Could you speak to this point: should Medicare really
1136 be paying for products that have no real value or paying more
1137 for products that have no added value? How do we balance a
1138 desire for rapid adoption of new technologies with ensuring

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1139 | that providers can be confident in the safety and benefit of
1140 | new technologies as they are held accountable for their use?

1141 | Mr. JACQUES. New technologies remind me of teenagers,
1142 | and both of my children are adults, so I survived raising
1143 | teens. We see glimpses of their future promise, but we also
1144 | recognize that not all of them are going to be good drivers
1145 | as soon as we put them behind a wheel. As a society, we
1146 | accept this and we balance their independence with our risks
1147 | through a variety of mechanisms, whether it is a learner's
1148 | permit or a prohibition on consuming alcohol or driving with
1149 | friends.

1150 | I believe in an ideal health technology system. We
1151 | would have one where lessons are learned quickly and
1152 | disseminated broadly. That depends on reliable collection,
1153 | analysis, and publication of real world data that arise from
1154 | using patients who are more typical than those studies in
1155 | pivotal trials and who are treated in their communities by
1156 | their own physicians.

1157 | Mr. WAXMAN. Okay. But what does that mean if a doctor
1158 | wants to use a new technology, and he has to be confident
1159 | that this is going to be safe and it is going to benefit the

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1160 patient?

1161 Mr. JACQUES. I am sorry if I was obtuse. What I was
1162 trying to convey is that the timing of calling the question
1163 is as critical as the content of the question itself. And
1164 especially for new technologies, the issue is being asked to
1165 call the question arguably prematurely to give it a thumb's
1166 up or a thumb's down when, in fact, what you actually have is
1167 an adolescent technology that has promise, but you do not
1168 really know the final answer.

1169 Mr. WAXMAN. And should we be paying for that through
1170 the Medicare system when we do not know whether it is going
1171 to add any value to what we already have available to us?

1172 Mr. JACQUES. There are people who feel strongly on
1173 both sides of that question, sir.

1174 Mr. WAXMAN. So when we do we call the previous question
1175 to get their vote?

1176 Well, we hear a lot of concern raised from manufacturers
1177 on the cost of data collection to the healthcare system both
1178 in real terms and in delays of bringing new technologies to
1179 patients. However, as you suggest under the 510(k) paradigm,
1180 some devices may be cleared for marketing with no relevant

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1181 | clinical trial evidence at all. Could you discuss your
1182 | concerns with that program and the potential risk to the
1183 | healthcare system of Medicare covering such technologies even
1184 | under its coverage with evidence development authority?

1185 | Mr. JACQUES. Yes. While that paradigm is appropriate
1186 | for many low-risk devices, I would focus my own attention on
1187 | that subset of cleared devices where untested claims of
1188 | enhanced benefit are made beyond the predicate device, or
1189 | where subsequent evidence may raise questions about the
1190 | fundamental impact of the technology.

1191 | I think the premise of the 510(k) program makes it more
1192 | difficult for a sponsor to articulate an enhanced value
1193 | proposition for a technology when it has been found to be
1194 | substantially equivalent to an old technology.

1195 | And that to me is the critical point in terms of paying
1196 | for value. That value proposition that you are essentially
1197 | better than something is hard to make if you have not
1198 | actually been compared to anything else.

1199 | Mr. WAXMAN. So we may have a 510(k) to get the device
1200 | approved, but we ought to know before we start paying a lot
1201 | of money for it that it is going to work.

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1202 Mr. JACQUES. Yes, sir.

1203 Mr. WAXMAN. Thank you. Thank you, Mr. Chairman.

1204 Mr. PITTS. The chair thanks the gentleman, and now

1205 recognizes the vice chairman of the subcommittee, Dr.

1206 Burgess, 5 minutes for questions.

1207 Dr. BURGESS. Thank you, Mr. Chairman. And I want to

1208 thank the ranking member for his generosity in mentioning

1209 H.R. 4015, which was the repeal of the sustainable growth

1210 rate formula, which did come through this committee. We are

1211 about at the 1-year anniversary of that 51 to zero vote.

1212 That was a landmark occasion for the committee. And in many

1213 ways, the development of that SGR policy was very similar to

1214 what is happening with the Cures Initiative. So I think that

1215 provides a template that ultimately could speak to success

1216 for the Cures Initiative that as we opened the doors up, we

1217 took information, asked for information from physicians, from

1218 patients, as to what they needed to see in the repeal of the

1219 sustainable growth rate. As a result, nobody got exactly

1220 what they wanted, but we got a product that was ultimately

1221 supportable by both Republicans and Democrats on this

1222 committee, and ultimately did pass the floor of the House,

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1223 | though we are still waiting on the Senate.

1224 | Ms. Grealy, I need to ask you a question about there is
1225 | a bill that Donna Christensen and I have done, H.R. 2663,
1226 | which deals with CBO scoring, because oftentimes it seems
1227 | like there are good ideas that are developed within the
1228 | healthcare sphere, but then CMS will say, but you know what?
1229 | All we can do -- or the CBO will say CMS just tells about the
1230 | cost, so all we can do is report to you on the cost. So the
1231 | ability to implement this new regimen is, in fact, a cost
1232 | driver for the system, and cannot be regarded as a cost
1233 | saver.

1234 | And, in fact, in this committee, even though I did not
1235 | support the Affordable Care Act, on this committee I
1236 | recognized a great deal of anxiety on the part of my
1237 | Democratic counterparts in dealing with Mr. Elmendorf at the
1238 | Congressional Budget Office. Wait a minute, we get no credit
1239 | for all of the savings we are going to get from treating
1240 | things in a more timely fashion.

1241 | So in your role at the Healthcare Leadership Council,
1242 | have you studied that issue at all?

1243 | Ms. GREALY. Yes, Mr. Burgess, and we strongly support

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1244 | the legislation --

1245 | Dr. BURGESS. I felt you would say that.

1246 | Ms. GREALY. You think so.

1247 | Dr. BURGESS. That is why I asked you.

1248 | Ms. GREALY. And delighted that it is bipartisan

1249 | legislation as well. But as you know, innovation plays a

1250 | strong role in wellness and prevention. And what our members

1251 | have seen are long-term savings when you make that investment

1252 | in wellness and prevention.

1253 | And as you point out, unfortunately CBO in their

1254 | traditional scoring methods does not give you credit for

1255 | those long-term savings. And we know that 70 to 80 percent

1256 | of healthcare costs today are going towards the treatment of

1257 | preventable chronic disease, and we know that if we make an

1258 | investment over the long term, we will see a dramatic

1259 | reduction in those healthcare costs. So your legislation

1260 | would not mandate that CBO have this longer scoring window,

1261 | but at least we would have that option so that you as members

1262 | of Congress could see that information and then make your

1263 | decision on making those investments, which may have a short-

1264 | term cost, but we know in the long term will result in better

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1265 health and lower costs for the healthcare system.

1266 Dr. BURGESS. Well, oftentimes it seems, you know, today
1267 we only end up talking about the costs of a therapy and we do
1268 not recognize the fact that, my goodness, we have beaten one
1269 of the big scourges of people's health. I mean, the
1270 hepatitis C treatment comes to mind. Instead of talking
1271 about the victory over hepatitis C, a disease that did not
1272 even have a name when I was in residency. We called it non-
1273 A/non-B hepatitis. And now there is a treatment for it that
1274 is, in fact, a cure. I mean, that is pretty big news.

1275 So, I mean, it is my hope that the Cures Initiative will
1276 be able to focus on those things. Yes, we will talk about
1277 price and we will talk about cost as we go through. But the
1278 big news, the headline is hepatitis C vanquished in our
1279 lifetimes, and that is a big deal.

1280 Dr. Schmizzi, I wanted to ask you a question on the
1281 Sunshine Act and the Sunshine Act provision that was
1282 contained in the Affordable Care Act. It does seem like it
1283 was written pretty broadly, and now the implementation is or
1284 runs the risk of hindering communication and information
1285 sharing amongst physicians.

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1286 So a rule that came out over the 4th of July weekend may
1287 prevent some of the country's most qualified physicians from
1288 giving lectures to fellow physicians through continuing
1289 medical education. Have you heard of providers that are
1290 having difficulty getting access to medical studies or even
1291 finding it more difficult to access continuing education
1292 because of the way this law is being implemented?

1293 Dr. SCHIMIZZI. Excuse me, Congressman. I do not hear
1294 of anything yet, but I can certainly see it coming that the
1295 Sunshine Act provision, the way it is written, can actually
1296 inhibit speakers from wanting to attend or be participants in
1297 a medical conference because of the information that will be
1298 published about them being paid and where the money comes
1299 from.

1300 Most institutions, most professional associations get
1301 their funding from member dues, but they also get funding
1302 from industry support in the form of gifts or donations. And
1303 those gifts and donations, if they are identified to be tied
1304 to CME credits, can actually impair the desire of
1305 academicians and thought leaders in medicine to give those
1306 presentations in front of those societies. So it can have a

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1307 | real negative impact on that. I do not believe it has
1308 | happened yet in my sub-specialty, but it is certainly is
1309 | possible.

1310 | Dr. BURGESS. Thank you for that answer. Mr. Chairman,
1311 | I hope that is something that this committee will keep in
1312 | mind and continue to monitor as we go forward. I will yield
1313 | back.

1314 | Mr. PITTS. The chair thanks the gentleman, and now
1315 | recognizes the gentleman from Texas, Mr. Green, 5 minutes for
1316 | questions.

1317 | Mr. GREEN. Thank you, Mr. Chairman. And again, thank
1318 | our witnesses for being here. A central component in
1319 | improving the quality of our healthcare system and developing
1320 | 21st century cures must be data-driven innovation. Mr.
1321 | Mussallem, in your testimony you talked about the coverage
1322 | for evidence development CED determination, how it can be
1323 | useful if used appropriately.

1324 | However, the challenge of ensuring CED is a tool for the
1325 | reimbursement system to give patients access to
1326 | groundbreaking therapies rather than the burden that
1327 | ultimately limit innovation remains before us. Can you tell

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1328 | us how we might be able to handle that?

1329 | Mr. MUSSALLEM. Sure. You know, particularly the use
1330 | of CED, I think, is valuable for therapies that are new and
1331 | really have not been evaluated in the past. In many cases,
1332 | the therapies that can be reimbursed are ones that are well
1333 | understood, and you could establish the safety and a safe and
1334 | necessary threshold. But in the case where you just do not
1335 | know much because they are novel, it is helpful to be able to
1336 | apply CED.

1337 | It is not always clear in the beginning of the CED
1338 | process exactly what evidence is going to be collected and
1339 | how much is necessary. And one of the things that is also
1340 | not clear about CED is when does it come to an end? At some
1341 | point in the initial stages of a technology, it is very
1342 | valuable to learn as much as you can and collect that
1343 | evidence. But once you have done that for some period of
1344 | time, it is appropriate for CED to sunset so that it does not
1345 | just become another layer of cost that sits on the healthcare
1346 | system.

1347 | And so, it is important, I think, to define CED more
1348 | thoughtfully and carefully as we think about using it as a

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1349 | tool. But it has great promise for entering areas that are
1350 | unknown.

1351 | Mr. GREEN. Okay. Do you have any mechanisms you would
1352 | suggest to enhance the coverage of these innovative
1353 | therapies?

1354 | Mr. MUSSALLEM. It is not a simple question. You know,
1355 | in the case of trans-catheter technology, CED was used, and
1356 | it was used that allows for this important aspect of medical
1357 | device development to be evaluated, different than a drug.

1358 | Medical technology is one that it an iterative process.
1359 | Because we make tools for physicians, often we get a lot of
1360 | feedback from physicians and they say, could you make it
1361 | better. Could you make it make it smaller? Could you make
1362 | it do things that it does not do today? And we respond to
1363 | that. And through those changes, therapy improves
1364 | dramatically.

1365 | And so, a coverage evidence development tool that is
1366 | flexible, and this is what was done in the case of trans-
1367 | catheter heart valves, allowed for the system to accommodate
1368 | new generations, new indications, as the evidence supported
1369 | it. So that is a powerful use of that tool.

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1370 Mr. GREEN. Dr. Jacques, is there any other mechanism
1371 available to provide coverage to these innovations?

1372 Mr. JACQUES. There are other mechanisms, including
1373 regulations concerning Medicare coverage for FDA-approved
1374 Category B investigational device exemption trials, the
1375 challenge being that aside from CED and those IDEs, there is
1376 not an obvious path for other sorts of valuable research.

1377 Mr. GREEN. Okay. Anyone else on the panel for those
1378 issues or those questions?

1379 If not, our entire healthcare system is shifting to a
1380 model that embraces shared decision making by informed
1381 patients whose views are valued and considered at every stage
1382 of the treatment. We have heard a great deal about the
1383 potential value for patient preference information and
1384 regulatory risk benefit determinations, particularly in the
1385 context of medical device pre-market approval.

1386 The FDA has emphasized that patient tolerance for risk
1387 and perspective on benefit is an important consideration. It
1388 makes sense for the innovators and regulators to consider
1389 patient perspectives as they develop and evaluate medical
1390 devices.

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1391 Ms. Mussallem, again, what potential benefit do you see
1392 from incorporating patient preference information in
1393 regulatory determinations, and do you have any suggestion on
1394 how it could be incorporated in the process?

1395 Mr. MUSSALLEM. Sure. All medical technology and all
1396 medical advancements are not created equal. Some can have a
1397 profound patients' lives. In our case, sometimes it is the
1398 only difference between life and death for these patients.
1399 So when you are making that sort of a consideration as a
1400 regulator, you would you really love that the regulators,
1401 they have the ability to apply a risk benefit analysis when
1402 they are thinking about what they should do in terms of
1403 allowing this technology to come to patients.

1404 If you keep the bar too high in the pre-market approval
1405 setting, what you might do is in an effort to achieve great
1406 science, again allow patients to not benefit and, in fact,
1407 die or live very poor quality of life. And sometimes it
1408 would make some sense to allow a certain element of risk,
1409 certainly to safeguard against safety concerns, and have a
1410 basic level of evidence, but to study in a post-market
1411 setting the true depth of efficacy in a real-world setting,

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1412 and then apply that and make adjustments.

1413 So this is one that you would not want to be unfettered,
1414 but to give regulators, in effect, not only the ability to,
1415 but the mandate to take a risk benefit analysis I think would
1416 be a powerful enhancement for the system and make it a
1417 learning system rather than what we have today.

1418 Mr. GREEN. Thank you, Mr. Chairman.

1419 Mr. PITTS. The chair thanks the gentleman, and now
1420 recognizes the gentleman from New Jersey, Mr. Lance, for 5
1421 minutes for questions.

1422 Mr. LANCE. Thank you, Mr. Chairman, and good
1423 afternoon to you all. Ms. Grealy, in your testimony you
1424 state that the key to harnessing the potential of real time
1425 data lies in putting the policies and practices in place that
1426 allow us to harness this data. You then go on to state the
1427 importance of protecting confidential health information
1428 while also making data appropriately accessible under HIPAA.

1429 In several of our recent hearings, witnesses have
1430 pointed out the challenges that arise in ensuring that this
1431 innovative technology is HIPAA compliant. Are there ways in
1432 which HIPAA inappropriately restricts the sharing and use of

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1433 patient data by researchers and medical providers?

1434 Ms. GREALY. Well, I think it is an ongoing challenge.

1435 And really what are trying to do is find the appropriate

1436 balance between protecting that patient information, but not

1437 stifling the innovation or access. And so we constantly have

1438 to keep that in mind.

1439 And periodically proposals are put forward that really

1440 would consume a lot of resources and time, and really do not

1441 create any particular value for the patient. I will use an

1442 example of that disclosure of, you know, everyone who has had

1443 access to the patient information, whether they are within

1444 that healthcare operations that I discussed, which is

1445 reasonably expected by patients.

1446 So I think it is all about making sure that we do not

1447 try to micromanage this, and we really put the patient at the

1448 center of it. And how can we create better value for that

1449 patient? And so, as we are looking at new ways and new

1450 access to information, and I will use as an example of that,

1451 as I mentioned earlier, patient engagement and the new tools

1452 for that, the mobile apps.

1453 And we are spending a lot of time, those of us that have

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1454 | been minding the HIPAA world for many, many years, working
1455 | with those app developers and telling them, you know, as you
1456 | are approaching this, we do not want to hinder your
1457 | innovation, but try to build into your system up front the
1458 | appropriate patient protections and information protections.
1459 | But again, the key is let us not stifle that innovation by
1460 | them, and let us not defer a whole lot of resources that
1461 | could be going towards patients care and treatment and
1462 | innovation by getting caught up in too much compliance
1463 | activity.

1464 | Mr. LANCE. Is there something we should be doing here
1465 | in Congress to make this a better situation?

1466 | Ms. GREALY. I would almost say do not do too much.

1467 | Mr. LANCE. First do no harm.

1468 | Ms. GREALY. Yes, first do no harm.

1469 | Mr. LANCE. To coin a phrase.

1470 | Ms. GREALY. Yes. And as we are looking, we have heard
1471 | a lot today about registries and how we can use that
1472 | information. I would say, again, let us not stifle that
1473 | access and the use of that information.

1474 | And the other very powerful thing that we are seeing

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1475 | that I think is going to be make all of this much more
1476 | available and much more usable is what is happening with
1477 | health information technology. I do not think any of us
1478 | could have imagined even 5 years ago how rapidly we are able
1479 | to build these databases and how rapidly we are not able to
1480 | access that information. And more importantly, get those
1481 | best practices to the physicians right as they are treating
1482 | the patients and having those practice guidelines, which is
1483 | going to go a long way towards creating that value that we
1484 | have all talked about in our healthcare system.

1485 | Mr. LANCE. Thank you. The Physician Payments
1486 | Sunshine Act, usually known as the Sunshine Act, requires
1487 | manufacturers of drugs and medical devices that participate
1488 | in Federal health programs to report payments to physicians
1489 | in teaching hospitals. In your judgment, has that data
1490 | sharing in this regard been beneficial to medical innovation?
1491 | Ms. Grealy?

1492 | Ms. GREALY. Again, I would sort of caution, and I
1493 | think we heard a lot today on this panel. We all believe in
1494 | transparency. We think that is important, and having the
1495 | disclosures about, you know, collaborations between physician

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1496 and industry.

1497 What we are most concerned about, and what we have
1498 actually seen already is the chilling effect, that physicians
1499 are concerned. Wait a minute, you know, this is a minimum
1500 amount of money. It is just not worth it to have my name on
1501 a list when there is no context about what was the value of
1502 that collaboration.

1503 And I think you heard Mr. Mussallem talk about their
1504 interactions with physicians as they are developing new
1505 cures, new devices. It is absolutely critical. And their
1506 partnerships with academic health institutions, absolutely
1507 critical.

1508 So again, it is about balance. We think there should
1509 be, you know, reporting this information, but it needs to be
1510 in context so that people know what is the value of having
1511 physicians working with manufacturers, and how does that
1512 benefit patients.

1513 Mr. LANCE. Thank you. My time has expired. Thank
1514 you, Mr. Chairman.

1515 Mr. PITTS. The chair thanks the gentleman, and now
1516 recognizes the gentleman from Florida, Mr. Bilirakis, 5

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1517 minutes for questions.

1518 Mr. BILIRAKIS. Thank you, Mr. Chairman. I appreciate
1519 it very much. First question is for Dr. Schmizzi. There are
1520 about 7,000 diseases and only 500 treatments available.
1521 Patients with rare diseases frequently have no approved
1522 treatments. This forces these patients to find off label
1523 usage for medication to treat their condition. If the FDA
1524 has rules limiting information to doctors and patients, this
1525 could harm a patient's health. And I know this was touched
1526 on earlier.

1527 Our health system should be patient-centered type of
1528 care, in my opinion. Given that, how can we ensure that
1529 patients and their physicians have access to information,
1530 whether it be on label or off label uses, so that it can
1531 determine the best course for treatment?

1532 Dr. SCHIMIZZI. Thank you, Congressman. I think the
1533 best way to do that is to ask the FDA or direct the FDA by
1534 legislation, or statute, or regulation changes, to allow that
1535 communication to go forward. Right that now communication is
1536 badly stifled, and much of the information that
1537 pharmaceutical manufacturers and innovators have is hidden

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1538 | from our view.

1539 | Rheumatology treats many diseases that have no defined
1540 | treatment. There is no medication that is defined to treat
1541 | Sjogren's syndrome. There is not defined treatment, no
1542 | medication specifically defined to treat chondrocalcinosis.
1543 | Some of these unusual diseases that are not really rare. We
1544 | see a lot of people with that, but we have no defined
1545 | mechanism or medicine that is approved for the use in these
1546 | diseases.

1547 | But things like Sjogren's syndrome, I am certain that
1548 | the pharmaceutical company that has manufactured some of the
1549 | medications available today have had crossovers with patients
1550 | who have Sjogren's syndrome, and they have data on how those
1551 | patients' Sjogren's symptoms improved or worsened, which is
1552 | also important to know. Did a particular medication make
1553 | that particular subset of symptoms worse?

1554 | Those things are important for us to know, but those
1555 | things may not be readily available to us. And those would
1556 | be very helpful to have.

1557 | Mr. BILIRAKIS. Thank you. Would it improve the
1558 | standard of care to have these indications on the label, such

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1559 as ensuring correct dosage and access to insurance
1560 reimbursement? And should we incentivize sponsors to do the
1561 additional studies to get these off label uses on label?

1562 Dr. SCHIMIZZI. I think incentivization to do some of
1563 these studies on these small diseases would be very, very
1564 helpful. It took 15 years to define that dermatomyositis was
1565 treatable with a medication that has been on the market for
1566 20 years. It took that long to get a large enough sample
1567 size to prove that the medication really worked. And
1568 dermatomyositis is a devastating inflammatory disease of the
1569 muscle that destroys muscle tissue and skin.

1570 So incentivizing those types of things would go a long
1571 way. And the National Institutes of Health already does
1572 that, and they were the ones who sponsored the actual study
1573 on dermatomyositis. But incentivizing manufacturers to go
1574 forward with some of these smaller diseases would be very
1575 helpful, yes.

1576 Mr. BILIRAKIS. Very good. Thank you very much. Mr.
1577 Mussallem, you mentioned that it was 4 years after the EU
1578 approved before the FDA approved the SAPIEN valve. Is the
1579 U.S. typically behind the EU for device approval? Why, and

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1580 | how can we accelerate the process?

1581 | Mr. MUSSALLEM. Yes, it was 4 years' difference. I
1582 | would say generally in medical devices and medical
1583 | technology, manufacturers would introduce their products
1584 | first in Europe. The burden of proof to be able to introduce
1585 | in Europe is much lower than the burden of proof required by
1586 | the FDA.

1587 | There is a level of safety that needs to be established
1588 | in Europe, but a much lower level of efficaciousness that is
1589 | required that is required before it moves to the marketplace.
1590 | And it is left to the judgment of physicians and patients on
1591 | whether it should be used, and the FDA requires a much higher
1592 | level of science to bring it to the United States.

1593 | Mr. BILIRAKIS. How can we accelerate the process here
1594 | in the United States?

1595 | Mr. MUSSALLEM. Well, there are several ideas, and a
1596 | number of them have actually been mentioned by Jeff Shuren,
1597 | who is responsible for CDR-8, including trying to think more
1598 | carefully and take a risk benefit analysis, and think
1599 | carefully about what might be collected in a pre-market
1600 | setting versus a post-market setting.

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1601 In the cases where patients really need the benefit, if
1602 you were to have a pre-market setting that was not so
1603 onerous, but rather have more extensive study in the post-
1604 market setting, what you could do then is potentially speed
1605 these cures to people that really need it when in the
1606 judgment of FDA it was the right thing to do. And at the
1607 same time, make sure that you are collecting the evidence so
1608 that therapies that are winners get supported and losers get
1609 stopped.

1610 Mr. BILIRAKIS. Well, thank you very much. Appreciate
1611 that. And I yield back, Mr. Chairman.

1612 Mr. PITTS. The chair thanks the gentleman, and now
1613 recognizes the gentlelady from North Carolina, Ms. Ellmers, 5
1614 minutes for questions.

1615 Mrs. ELLMERS. Thank you, Mr. Chairman. And thank you
1616 to our panel, especially Dr. Schimizzi -- welcome -- from
1617 Wilmington, North Carolina, one of my very favorite places
1618 and just down the road from Dunn, North Carolina.

1619 Dr. Jacques, I have a question for you. You state that
1620 CMS needs unambiguous authority to review clinical trials
1621 when claims related to these trials will be submitted for

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1622 Medicare payment. In what ways is CMS authority in this
1623 respect limited now, and how does this impact the search for
1624 cures?

1625 Mr. JACQUES. At a fundamental level, one would expect
1626 that the Medicare program or any insurance company would know
1627 what it is paying for as opposed to paying blindly. And my
1628 understanding is periodically Congress asks Medicare how much
1629 research are you paying for, and my understanding is the
1630 Agency has been unable to actually produce a number. So that
1631 would be helpful to know.

1632 I think that Medicare engagement on research would
1633 actually serve a number of purposes because I have fond much
1634 to my own frustration while I was a civil servant that there
1635 would often be times when companies would have come in with
1636 the data that they had, and we would sit there around the
1637 table going, you know, if only 2 years ago you had made this
1638 small change it would have made a very large course direction
1639 in where you came up.

1640 So the challenge is that Medicare covers routine costs
1641 in clinical trials based on a White House executive order
1642 from the end of the Clinton Administration. There is then a

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1643 distinct regulation on FDA Category B investigational device
1644 exemptions, and then there is CED. And in any particular
1645 trial, there may be an overlap of those things, so CED would
1646 include routine costs, for example. There may be CED that
1647 might also be combined in the context of an FDA IDE approval
1648 study.

1649 And because all of these things are siloed, it is very,
1650 very difficult at the staff level when a prospective
1651 investigator comes in and says, okay, here is my clinical
1652 trial. These are all the things I want to do. Can you tell
1653 me if it is covered or not. And that can be a conversation
1654 that takes months to get to all the details.

1655 And I believe that if CMS simply had a singular
1656 authority that would relate to this, it could then publish an
1657 actual integrated policy where all the pieces actually fit,
1658 and you were not running all over the place trying to find
1659 different parts of an answer.

1660 Mrs. ELLMERS. Mr. Mussellum and Dr. Schimizzi, it
1661 looked like you were very intrigued by Dr. Jacques' answer.
1662 Is there anything that either one of you would like to
1663 comment on?

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1664 Mr. MUSSALLEM. Yes. We think that just by listening
1665 to comments of Dr. Jacques and others in CMS, we have heard
1666 that there are limitations to what policy allows CMS to do.
1667 And also that there are limitations associated with their
1668 staffing levels, and that is concerning to us. We are
1669 dependent on payment to be able to bring these technologies
1670 to patients.

1671 You know, one of their particular things that are most
1672 noteworthy is much of the great medical breakthroughs come
1673 from individuals, or very small companies, or somebody that
1674 just has a great idea. And being able to take that from a
1675 napkin to reality is becoming longer and longer and more and
1676 more costly.

1677 And to be able to have a conversation with CMS that
1678 clearly defines a predictable process would be very powerful
1679 to those organizations. And this unpredictability has a
1680 chilling effect on innovation, so that kind of clarity would
1681 be very positive.

1682 Mrs. ELLMERS. Dr. Schimizzi?

1683 Dr. SCHIMIZZI. I have found that what Dr. Jacques
1684 mentioned about staffing problems being an issue with

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1685 national coverage determinations and local coverage
1686 determinations, contrasting one another, conflicting, very
1687 intriguing especially because that is a topic that has really
1688 hit us very hard this last year when we have a patient who
1689 may live in North Carolina part of the year and New York
1690 another part of the year, and they have Medicare. They may
1691 be able to get the medication in North Carolina, but when
1692 they go to New York the medication is denied because the
1693 carriers are different and the coverage determination is
1694 different.

1695 It would be really nice to have a uniform set of rules.

1696 Mrs. ELLMERS. Centralized.

1697 Dr. SCHIMIZZI. Yes. I mean, that is essentially what
1698 the national coverage determination was meant to do. But I
1699 can now understand, given what I have heard today, that it
1700 might indeed be a staffing problem that does not allow CMS to
1701 act on the national level, and allows individual carriers to
1702 make different determinations in different States, which
1703 makes it difficult for a patient to get the same care in
1704 different areas of the country.

1705 Mrs. ELLMERS. Right. Well, thank you. And my time is

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1706 | about expired, so I will leave it at that. Thank you, Mr.
1707 | Chairman.

1708 | Mr. PITTS. The chair thanks the gentlelady, and now
1709 | recognizes the gentlelady from Colorado, Ms. DeGette, 5
1710 | minutes for questions.

1711 | Ms. DeGETTE. Thank you very much, Mr. Chairman, and
1712 | thank you again for your commitment to these hearings.

1713 | Mr. Mussallem, in your written testimony you mentioned
1714 | some of the advantages of registries to help with post-market
1715 | surveillance. And you talk about the American College of
1716 | Cardiology and the Society of Thoracic Surgeons working
1717 | collaboratively to create the STSACCTVT registry. Can you
1718 | talk to us a little bit more about that registry, when it was
1719 | formed, the cost, and who can access that data and
1720 | information?

1721 | Mr. MUSSALLEM. Yes, thank you. It was a remarkable
1722 | collaboration. And again, routinely when a new technology is
1723 | approved, FDA would mandate a post-approval study. In this
1724 | case, the idea of mandating a post-approval study took a
1725 | couple of forms, and an alternative was presented to collect
1726 | data in a registry rather than collect more extensive data on

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1727 | a smaller group of people.

1728 | Through conversations with CMS -- as a matter of fact,
1729 | this actually became on where CMS also became partners in
1730 | this discussion as well as a number of other stakeholders.
1731 | It actually became part of the national coverage
1732 | determination because the national coverage determination
1733 | said we will pay for this new procedure if you are collecting
1734 | evidence. And they did that under the provisions of coverage
1735 | with evidence development.

1736 | So this registry became multi-purpose, and it did a few
1737 | things. One is it became the post-approval study for FDA and
1738 | to follow patients on a long-term basis with this new
1739 | therapy, and every patient gets this, so it is a very large
1740 | and powerful database. It became the tool used for evidence
1741 | collection for CMS in terms of their ultimate decision on
1742 | coverage for evidence development. And it just became very
1743 | powerful to the medical community because here was a set of
1744 | data that rather than being managed by a company --

1745 | Ms. DeGETTE. Excuse me. Excuse me. They only give me
1746 | 5 minutes.

1747 | Mr. MUSSALLEM. I am sorry.

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1748 Ms. DeGETTE. And I appreciate every minute of that.
1749 Who can access that data and information from that registry?

1750 Mr. MUSSALLEM. Yes. That is actually where I was
1751 going.

1752 Ms. DeGETTE. Okay.

1753 Mr. MUSSALLEM. This data is managed by the American
1754 College of Cardiology and the Society of Thoracic Surgeons as
1755 a matter of fact. So it is housed within their organization,
1756 and so they have access to it. There is an advisory board
1757 that includes many of the members of those societies that
1758 actually ride herd over that data and publish results from
1759 that data on a routine basis.

1760 Ms. DeGETTE. Okay. Now, there are some limitations, I
1761 think, that you and others have pointed out with registries.
1762 And I am wondering, do you think it is just because we do not
1763 have a lot of experience with it?

1764 Mr. MUSSALLEM. I think there is concern that we do not
1765 have experience with registries, and that is certainly true.
1766 We have no experience, for example, in our field of heart
1767 valves.

1768 Ms. DeGETTE. Right. Right. So we just need to learn.

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1769 | Dr. Rising, I wanted to ask you quickly along these lines, in
1770 | your testimony you talked about the Sentinel Initiative as a
1771 | possible alternative or supplement to registries. Can you
1772 | talk about how those can work for data collection?

1773 | Dr. RISING. Sure. I would be happy, thanks. So
1774 | Congress instructed FDA to establish the Sentinel Program in
1775 | 2007 to proactively monitor for problems with drugs and
1776 | biologics on the market. And in 2012, Congress instructed
1777 | FDA to expand Sentinel to include medical devices.

1778 | Now, what Sentinel is it uses claims data, almost
1779 | exclusively claim data, housed by payers to look for
1780 | associations between exposure to a particular product and
1781 | then a particular health outcome.

1782 | Now, Sentinel could be expanded to devices except that
1783 | right now there is no specific information on a device that
1784 | is used in care on the claims form. So a payer might have
1785 | information that they did a hip replacement that they are
1786 | paying for, but they have no information on the specific hip
1787 | replacements that were used.

1788 | Ms. DeGETTE. Right. Right.

1789 | Dr. RISING. So to expand Sentinel to include devices, a

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1790 new field needs to be placed on the claims form. And in
1791 general, and we are a big supporter of using existing
1792 structures, such as claims forms, to capture new information
1793 like this.

1794 Ms. DeGETTE. And, Mr. Mussallem, in your testimony you
1795 said that we need more resources and support for FDA. And I
1796 am wondering what types of resources you think we need. We
1797 have heard others talking about CMS. I am wondering about
1798 FDA.

1799 Mr. MUSSALLEM. You know, I think the leadership at FDA
1800 has a pretty clear vision of some things that need to change,
1801 and they have done a pretty good job of articulating that
1802 through their strategic plan.

1803 Ms. DeGETTE. Right. They have also told us about it,
1804 too. Thank you very much. Thank you, Mr. Chairman.

1805 Mr. PITTS. The chair thanks the gentlelady. I have a
1806 unanimous consent request. I would like to insert a letter
1807 from the Lupus Foundation of America into the record.

1808 Without objection, so ordered.

1809 [The information follows:]

1810

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

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1813 Mr. PITTS. This has been another very informative and
1814 important hearing. Thank you very much for testifying today.
1815 We will have a lot of follow-up questions I am sure from
1816 members. We will send those to you. We ask that you please
1817 respond promptly. Members will have 10 business days to
1818 submit their questions for the record. That means they
1819 should submit their questions by the close of business on
1820 Tuesday, August the 5th.

1821 Without objection, the subcommittee is adjourned.

1822 [Whereupon, at 4:39 p.m., the Subcommittee was
1823 adjourned.]