U.S. HOUSE OF REPRESENTATIVES
COMMITTEE ON ENERGY AND COMMERCE

October 1, 2017

TO: Members, Subcommittee on Health

FROM: Committee Majority Staff

RE: Hearing entitled "Examining Patient Access to Investigational Drugs"

I. INTRODUCTION

The Subcommittee on Health will hold a hearing on Tuesday, October 3, 2017, at 10:30 a.m. in 2322 Rayburn House Office Building. The hearing is entitled "Examining Patient Access to Investigational Drugs."

II. WITNESSES

Members will hear from four panels of witnesses.

Panel One:

- U.S. Rep. Brian Fitzpatrick (R-PA)
- U.S. Rep. Andy Biggs (R-AZ)

Panel Two:

- Scott Gottlieb, M.D., Commissioner, U.S. Food and Drug Administration (FDA).

Panel Three:

- John Dicken, Director, Health Care, U.S. Government Accountability Office (GAO).

Panel Four:

- Naomi Lopez-Bauman, Director of Healthcare Policy, Goldwater Institute;
- Lieutenant Commander Matthew Bellina, U.S. Navy (Retired);
- Kenneth I. Moch, President & CEO, Cognition Therapeutics, Inc.;
- Alison Bateman-House, PhD, MPH, MA, Assistant Professor, Department of Population Health, New York University (NYU) Langone Health; and,
- Ellen V. Sigal, Chairperson and Founder, Friends of Cancer Research.

III. BACKGROUND

The discussion will center on patient access to investigational drugs and devices. The U.S. Food and Drug Administration's (FDA) expanded access program is designed to help patients who do not qualify for clinical trials access unapproved therapies. At the same time, there is an ongoing movement in states to pass "Right to Try" laws, with the goal of removing perceived barriers terminally ill patients may face in accessing such treatments.

The U.S. Government Accountability Office (GAO) issued a report in July 2017 that reviewed investigational new drugs. According to this study, FDA approved 99 percent of nearly 5,800 expanded access requests from fiscal years 2012 through 2015.[1] Application response times ranged from hours for emergency single-patient requests to the allotted 30 days for other types of requests.

While this program is designed to benefit individuals with serious and life-threatening conditions, certain patients and patient groups have advocated for states to adopt "Right to Try" laws. To date, 37 states have a version of "Right to Try" laws, with the aim of allowing patients to access investigational drugs without first receiving FDA approval.

IV. ISSUES

The following issues may be examined at the hearing:

- FDA's steps to streamline the expanded access program;
- GAO's findings and recommendations for improving access to investigational drugs;
- ongoing enrollment challenges facing patients, patient advocates, and physicians;
- key contributors to manufacturers' review policies for participation in experimental drug programs; and,
- federal legislation, including:
    - S. 207, the "Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017," authored by Senator Ron Johnson (R-WI)(companion legislation in the House has been introduced by Rep. Fitzpatrick (R-PA) and Rep. Biggs (R-AZ); and,
    - H.R. 1020, the "Compassionate Freedom of Choice Act of 2017," introduced by U.S. Rep. Morgan H. Griffith (R-VA)
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[1] U.S. Government Accountability Office. "Investigational New Drugs: FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used." July 2017. Retrieved from: https://www.gao.gov/assets/690/685729.pdf

V. STAFF CONTACTS

If you have any questions regarding this hearing, please contact Adam Buckalew or Paul Edattel of the Committee staff at (202) 225-2927.