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Congress Boosts "Hope" for Infants with Rare Diseases

Josie Cooper

The Alliance for Patient Access (allianceforpatientaccess.org), founded in 2006, is a national network of physicians dedicated to ensuring patient access to approved therapies and appropriate clinical care. AfPA accomplishes this mission by recruiting, training and mobilizing policy-minded physicians to be effective advocates for patient access. AfPA is organized as a non-profit 501(c)(4) corporation and headed by an independent board of directors. Its physician leadership is supported by policy advocacy management and public affairs consultants. In 2012, AfPA established the Institute for Patient Access (IfPA), a related 501(c)(3) non-profit corporation. In keeping with its mission to promote a better understanding of the benefits of the physician-patient relationship in the provision of quality healthcare, IfPA sponsors policy research and educational programming.

Alliance for Patient Access

Institute for Patient Access

Congress needs to act to continue to spur new treatments for sick children with few options.

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The Creating Hope Reauthorization Act would extend an existing program (1) that incentivizes the development of treatments for rare pediatric diseases by offering a voucher for expedited FDA review. (2) Companies can then buy and sell these vouchers to capture a return on investment that rare drugs seldom deliver.

There have been 49 vouchers issued under the program, generated by the approval of treatments for 40 diseases impacting children, including a rare muscular dystrophy mutation (3), cystic fibrosis (4), and haemophagocytic lymphohistiocytosis. (5) No approved drugs (6) were available for most of these conditions before the incentives program existed.

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Research and Rare Diseases in Children

A complex cost-benefit equation applies to treatments for rare diseases.

Researchers and investors should prioritize technologies that can improve the well-being of children by addressing their unique diseases and challenges. The small number of patients affected by rare conditions makes it difficult to recover research and development costs, even if the treatment is wildly successful.

The difficulty of conducting clinical trials for children, the effects of delayed diagnoses, and a 15-year average for drug approval further complicate matters.

Fully 95% of rare diseases have no FDA-approved treatment. These medications serve a small patient population and are difficult to develop, test, and make available. The challenges are magnified for disease treatments for children, often discouraging traditional investment.

Patients and Providers Urge Congress to Reauthorize Vouchers

More than 600 patients, caregivers, providers, and advocates converged on the U.S. Capitol to tell their stories and plea for more public investment in policy solutions to healthcare challenges, just days after the Creating Hope Reauthorization Act was introduced. (7)

The bipartisan bill is sponsored by U.S. Representatives Gus Bilirakis (R-FL), Anna Eshoo (D-CA), Michael McCaul (R-TX), Lori Trahan (D-MA), Michael Burgess (R-TX) and Nanette Barragán (D-CA.). In remarks announcing (8) their sponsorship, several

representatives referred to personal acquaintances or constituents whose children live with rare diseases.

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Disclosure: *Josie Cooper is executive director of the Alliance for Patient Access. This article was also published at healthpolicytoday.org.*

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Corresponding Author

Josie Cooper
Executive Director
Institute for Patient Access
2020 K Street NW, Suite 505
Washington, DC 20006
Telephone: (202) 951-7095
Email: jcooper@woodberryassociates.com