

NORD[®]
National Organization
for Rare Disorders

PDUFA VII: Continuing Progress for Rare Disease Patients

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Alone we are **rare**. Together we are strong.[®]



NORD, an independent nonprofit, is leading the fight to improve the lives of **rare disease patients and families**.

We do this by supporting patients and organizations, accelerating research, providing education, disseminating information and driving public policy.



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Overview

1. CBER's Work On Cell And Gene Therapies Needs Additional Support
2. Rare Disease Cures Accelerator (RDCA)
3. Strengthening PDUFA VI's PFDD Efforts
4. Decentralized Clinical Trials

Realizing the Promise of Cell and Gene Therapies

Addressing CBER's Resource Needs in PDUFA VII

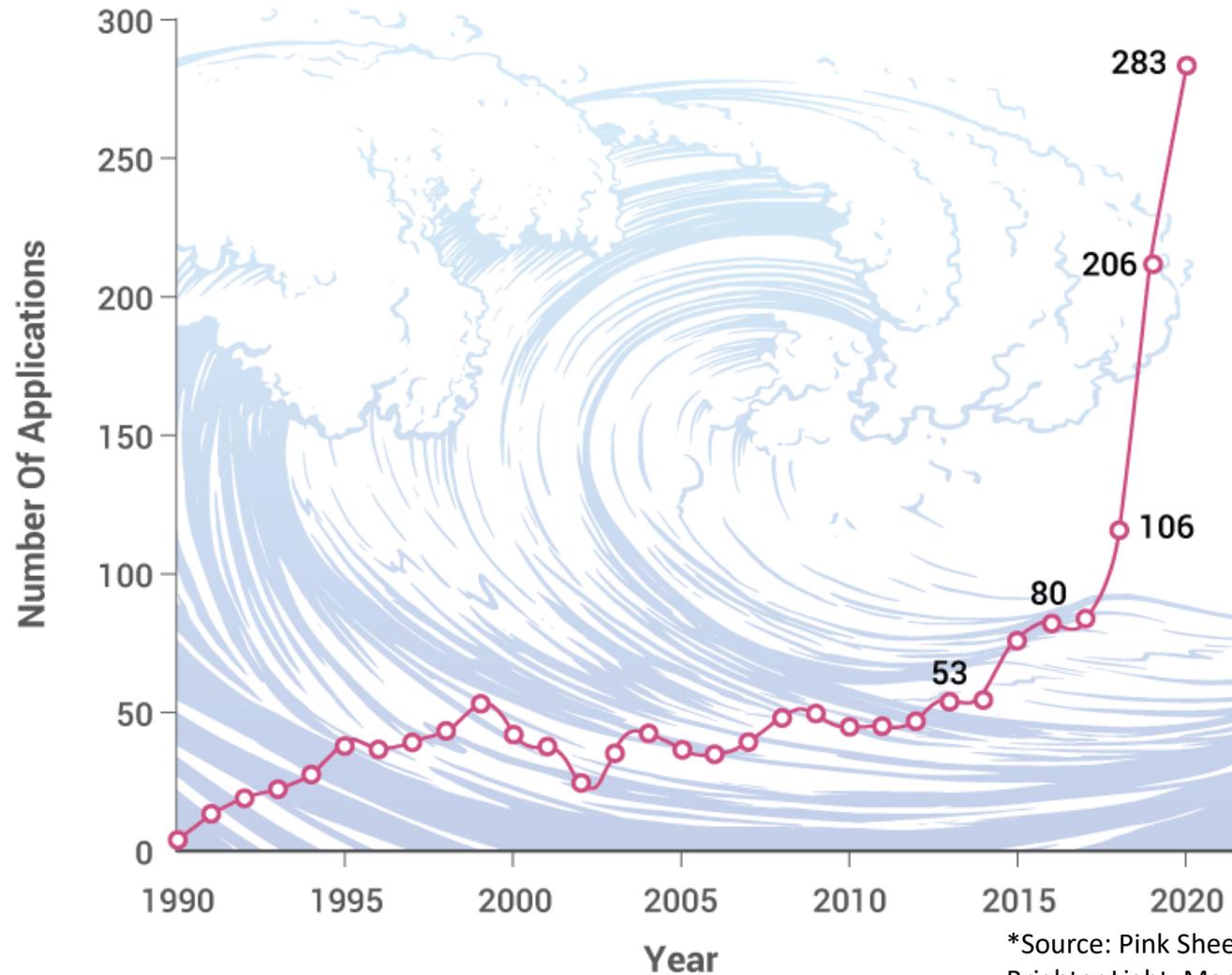


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Madilyn Yang (far left) has been battling a rare breathing disorder since birth called Central Congenital Hypoventilation Syndrome (CCHS) or Ondine's Curse.

Exponential Increase In CBER Workload



- 900 Active INDs at CBER
- 200 INDs/year
- Predicted 40-60 launches over next 10 years

*Source: Pink Sheet, Scientific Wave Pushes CBER Into Brighter Light, March 26, 2020



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Rare Disease Cures Accelerator

Critical work should be supported by
user fees to fully realize the potential
value of these programs



Vaughn family: Son, Morgan (left), diagnosed with Necrotizing Enterocolitis at four days old

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Overview of Rare Disease Cures Accelerator (RDCA) Initiative

"The Rare Disease Cures Accelerator would provide a more centralized infrastructure and common platform(s) and approaches to support:

(1) Rare disease characterization,

(2) development of standard core sets of clinical outcome assessments and endpoints relevant to rare conditions, and

(3) support conduct of clinical trials in rare disease populations."

*FDA, Rare Disease Clinical Trial Networks; Request for Information and Comments, June 1, 2020



Rare Disease Cures Accelerator- Data and Analytics Platform (RDCA-DAP)

Sep. 19, 2019

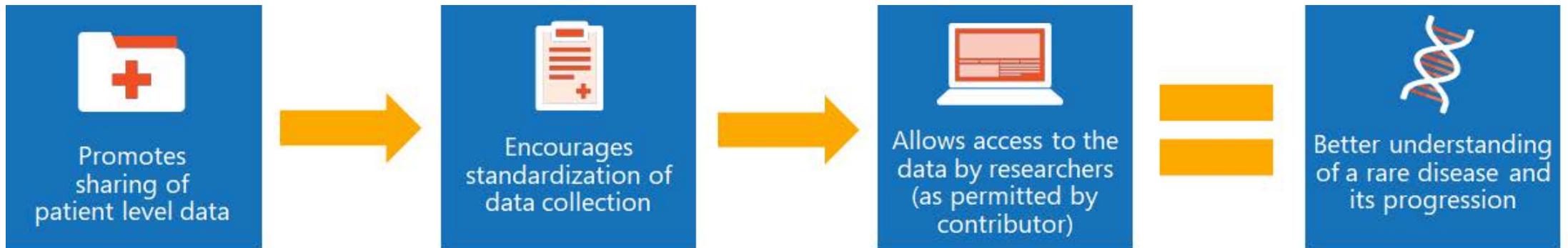
TOPIC: Press Releases, Featured News

Innovative Data and Analytics Platform to Accelerate Drug Development for Rare Diseases

Posted by Laura Mullen



Launched by C-Path and NORD through an FDA grant, the goal of the new platform is to accelerate the development of cures by addressing the need to better characterize rare diseases.



Clinical Outcome Assessments



C-Path @CPathInstitute · Mar 19



W/support from [@US_FDA](#), [#CPath](#)'s Patient-Reported Outcome Consortium and [@RareDiseases](#) are pleased to announce the development of a Rare Disease Clinical Outcome Assessment Consortium. bit.ly/393YO64 [#NORD](#) [#FDA](#) [#rarediseases](#) [#collaboration](#) [#consortium](#)



Northwestern



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Clinical Trials Network

- The Rare Disease Clinical Trials Network has the potential to benefit a wide array of stakeholders across the drug development spectrum.



FEDERAL REGISTER

The Daily Journal of the United States Government



(N) Notice

Rare Disease Clinical Trial Networks; Request for Information and Comments



PDUFA VII Should Build Upon PDUFA VI's PFDD Success



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*Stacy McCaroll and
daughter, Cystic Fibrosis*

Continued Progress in PFDD

PDUFA VII should provide for:

- Additional feedback mechanisms to sponsors and patients to communicate the role PED played in regulatory decision making
- Additional resources for early interaction with sponsors on PFDD plans





Decentralized Clinical Trials: Learnings from COVID-19



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Continued Progress on Modernized Clinical Trials is Necessary

- Under PDUFA VII, FDA should:
 - Issue guidance that enshrines aspects of telehealth/telemedicine that were successful during COVID-19
 - Advance Agency thinking around Decentralized Clinical Trials & Innovative Clinical Trial Design
 - Advance Agency thinking on Real World Evidence collection & use by reviewers
 - Issue guidance on incorporation of the use of digital technologies/tools in clinical trials





Thank you.



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