Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot

FDA's Rare Disease Day 2024

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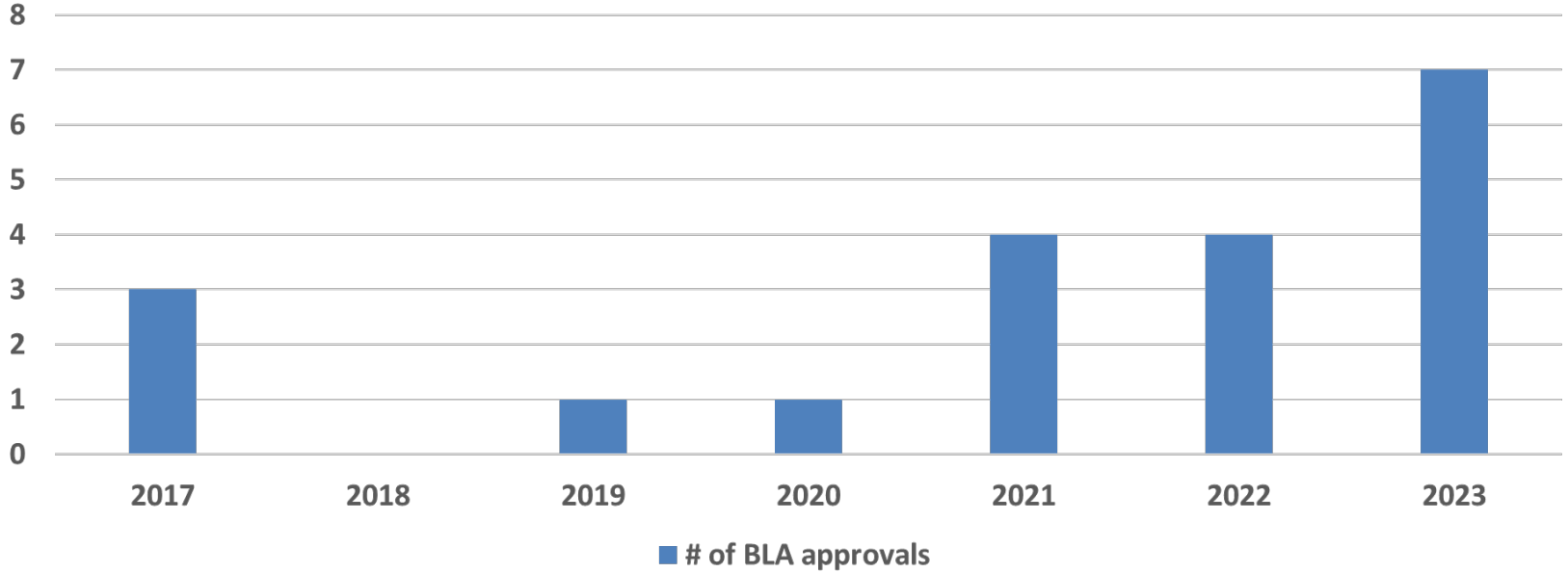
START Pilot Background

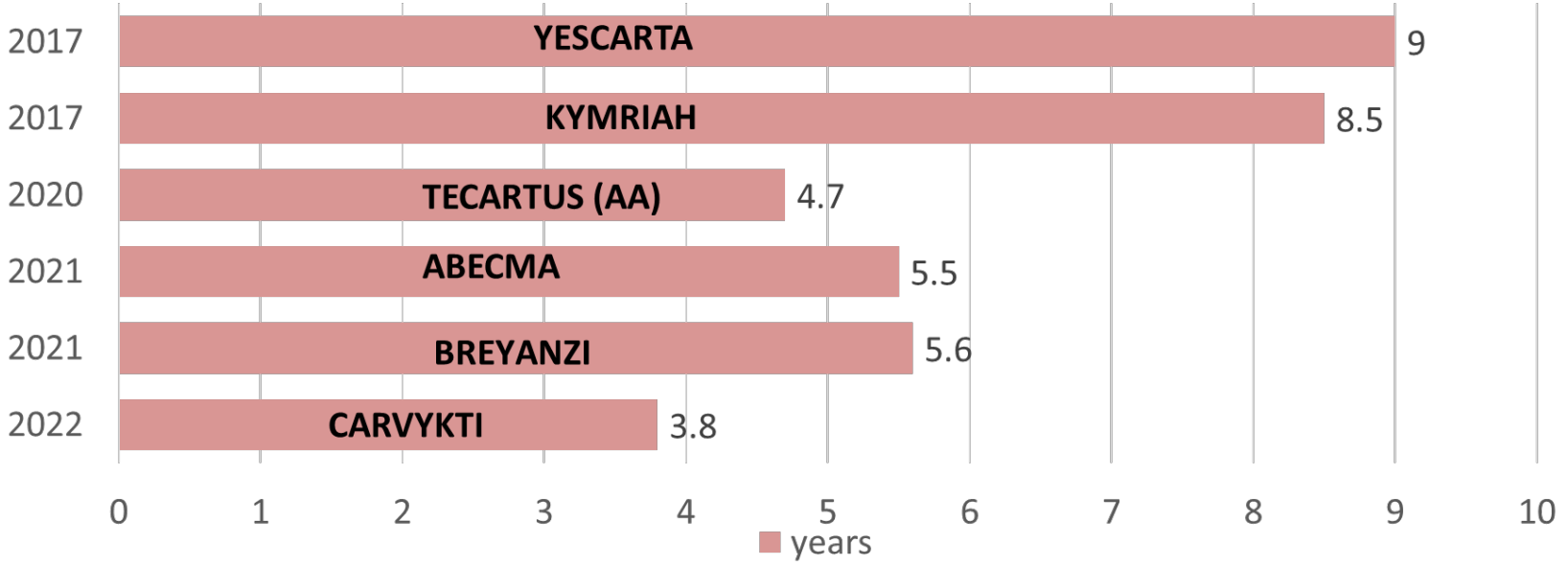
- CBER Office of Therapeutic Products and CDER Office of New Drugs jointly published a Federal Register Notice (FRN) announcing the START Pilot Program on 10/2/2023
- To further accelerate the pace of development of certain CBER- and CDER-regulated products that are intended to address an unmet medical need as a treatment for a rare disease
  - Formal meeting process can take weeks or months
  - The Pilot will test a “more rapid, ad-hoc communication mechanism”
  - Hypothesis: if reduction of waiting times could reduce the time to development

Cellular and Gene Therapy Products BLA Approvals for Rare Diseases



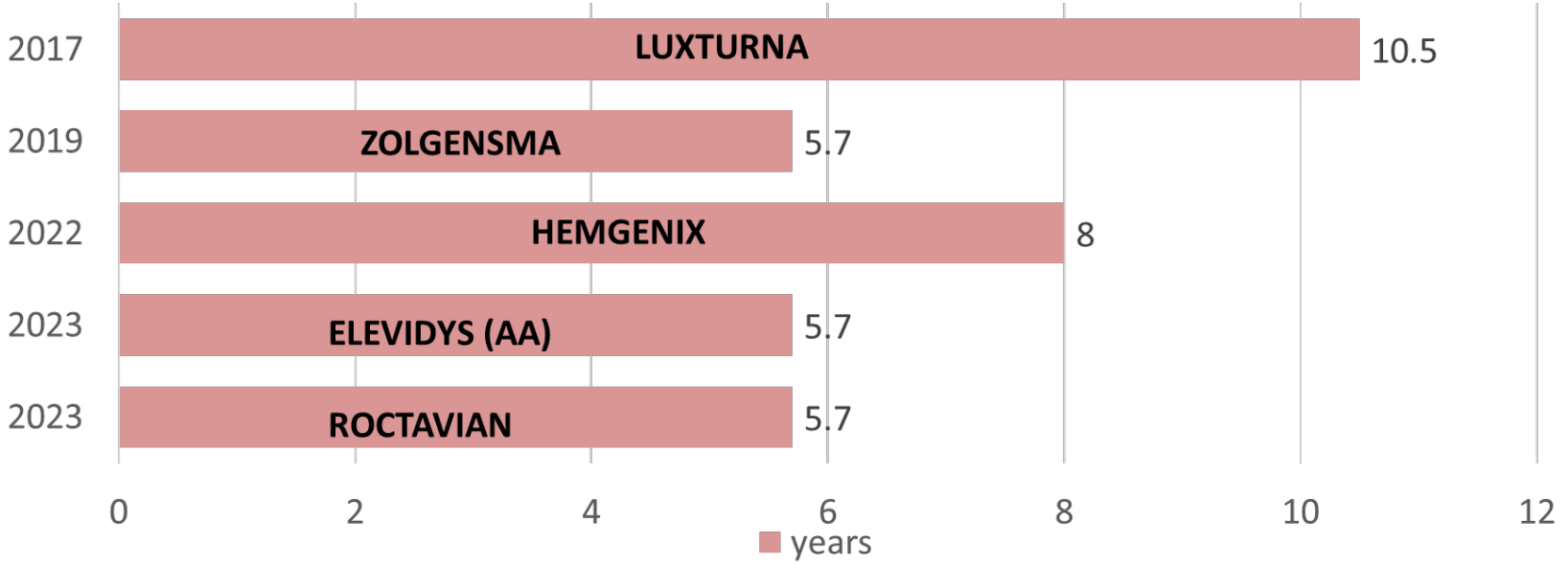
https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

CAR T Products: Time from IND Submission to Original BLA Approval



AA: Accelerated Approval

AAV Based Products: Time from IND Submission to Original BLA Approval



START Pilot – Federal Register Notice (FRN)

- Details of the pilot program
  - Background
  - Eligibility criteria
  - What to submit in a request to participate
  - Selection criteria and process
  - FDA / Sponsor interactions during pilot
- Initially select up to three participants in each Center
- A second iteration may be conducted to include more participants depending on the pilot program's success

Features of START Pilot Program

- Enhanced communications with FDA to address issues with specific programmatic needs for individual products
  - Additional communications beyond the currently available formal meetings
  - More frequent advice provided on accelerated timeline
- Potentially involving all review disciplines depending on the specific needs of individual programs
- Pilot is milestone-driven
  - Pilot considered concluded when the development program reaches a significant milestone such as initiation of the pivotal clinical study stage or to the pre-BLA or pre-NDA meeting stage as agreed upon with the sponsor

START Pilot Program Objectives

- To address development issues that would otherwise delay or prevent a promising novel drug from progressing to the pivotal clinical trial stage or pre-BLA/pre-NDA meeting stage
- To ensure a mutual understanding of information needed to facilitate initiating the pivotal clinical study or to get to the pre-BLA/pre-NDA meeting stage
- To help generate high quality and reliable data intended to support a BLA or NDA to facilitate approval efficiency

START Pilot Timelines

- Acceptance period for request to participate in pilot: January 2 – March 1, 2024
- CBER acknowledgment of the request: within 14 days of receipt of request to participate in pilot
- FDA notification to sponsors of acceptance into pilot by May 30, 2024

Enhanced Communications during the Pilot

- Initial meeting with pilot participants
  - Review features of the pilot
  - Overview the specific issues noted by the sponsor in their request to participate in the pilot
  - Discuss the proposed plan (e.g., format and frequency) for the subsequent ad hoc communications as part of the pilot program
  - Discuss the significant regulatory milestone (e.g., initiation of the pivotal clinical study or pre-BLA/pre-NDA meeting stage) when participation in the pilot will be considered concluded
- Subsequent communications
  - Ongoing interactions via email or teleconference
  - Take place on a scheduled and/or as needed basis

“We hope the insight gained from this pilot will provide information on how best to facilitate more efficient development of potentially life-saving therapies with rare disease indications and help sponsors generate high-quality, compelling data to support a future marketing application,” -- Peter Marks, M.D., Ph.D., director of the FDA’s CBER

“We share the goal of delivering potentially life-saving products to patients, and are committed to helping sponsors achieve regulatory milestones, while ensuring the safety, effectiveness and quality of these products.” -- Patrizia Cavazzoni, M.D., director of the FDA’s CDER

FDA Launches Pilot Program to Help Further Accelerate Development of Rare Disease Therapies | FDA

Contact Information

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- **Interactions with Office of Therapeutic Products website:**

  Interactions with Office of Therapeutic Products | FDA
- **OTP Learn Webinar Series:**

  https://www.fda.gov/vaccines-blood-biologics/news-events-biologics/otp-learn

FDA Headquarters

- **CBER website:** www.fda.gov/BiologicsBloodVaccines/default.htm
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