Elizabeth Glaser
Pediatric AIDS
Foundation

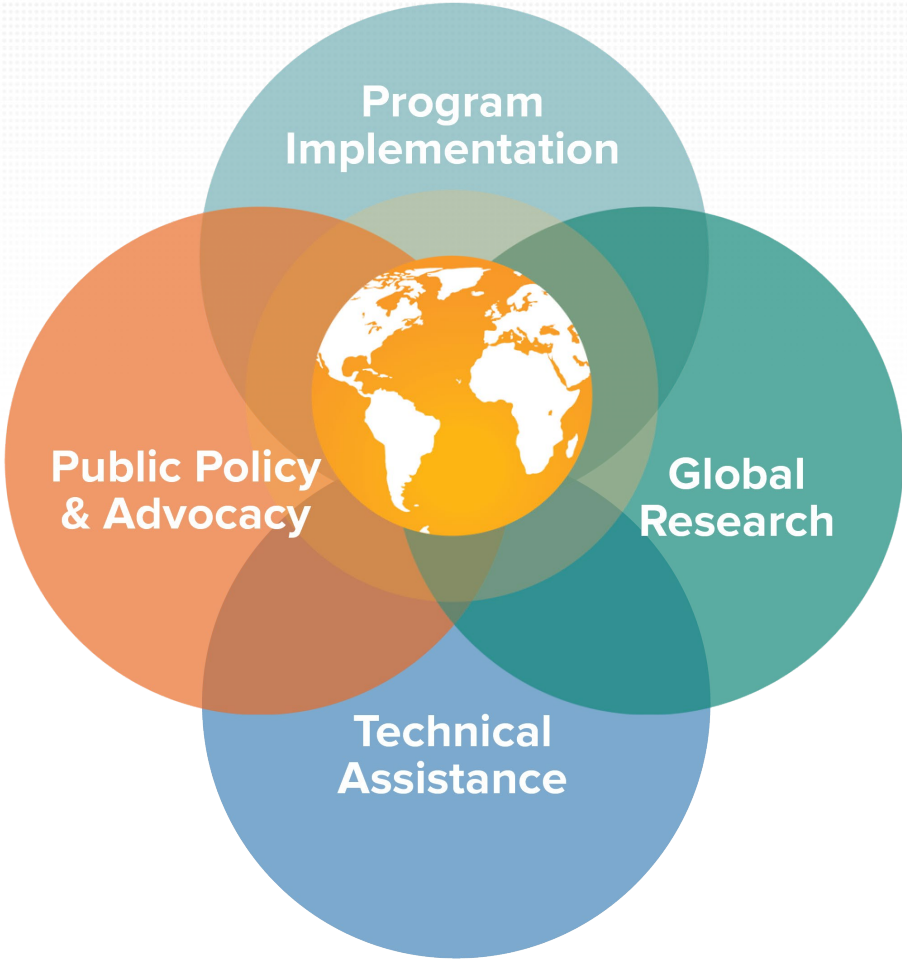
FDA Pediatric Stakeholder Meeting

Katie Coester, MPP | November 21, 2019

Advocating for Pediatric Medicines Goes to our Roots

- EGPAF's origins are rooted in Elizabeth's fight to get HIV medicines tested for use in children
- Longstanding commitment to promoting FDA reforms, including requirements and incentives for studying medicines in children
- As a global organization, EGPAF understands how U.S. policies on medicines impact children around the world

EGPAF Today: Integrated and Comprehensive Approach to Ending AIDS in Children

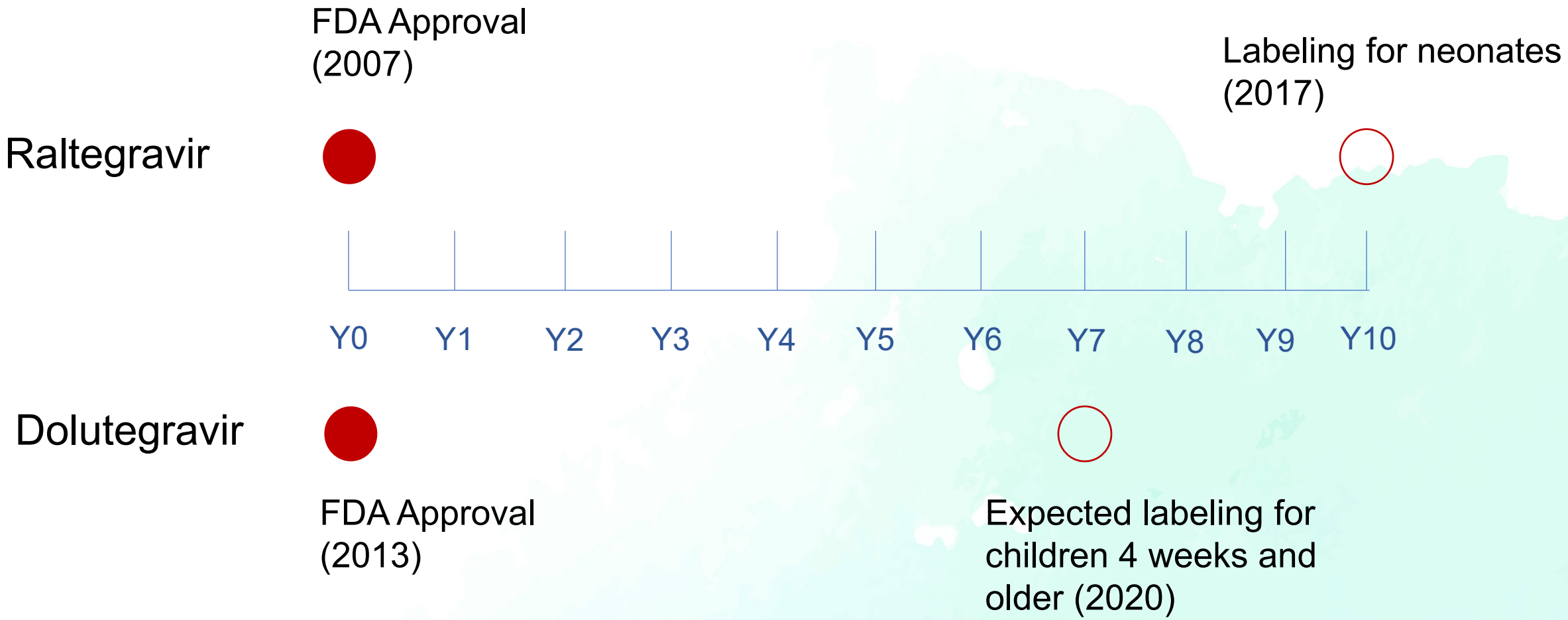


- 30 year history in research, advocacy, program implementation and technical assistance
- Supporting 5000+ sites around the world, with over 1.6 million individuals enrolled on ART as of Sept 2018
- Brings an on-the-ground perspective to addressing drug development and drug access challenges for treating HIV and other related diseases in children and adults

Significant Pediatric HIV Treatment Gaps Remain

- Smallest children are still using AZT – a medicine approved 32 years ago and no longer used by adults
- In the last ~20 years, **14** new individual ARV compounds have been approved for adult use, but many are not yet approved for children
- New pediatric formulations of HIV drugs are being rolled out in countries, but challenges of accessing them and scaling up their use remain

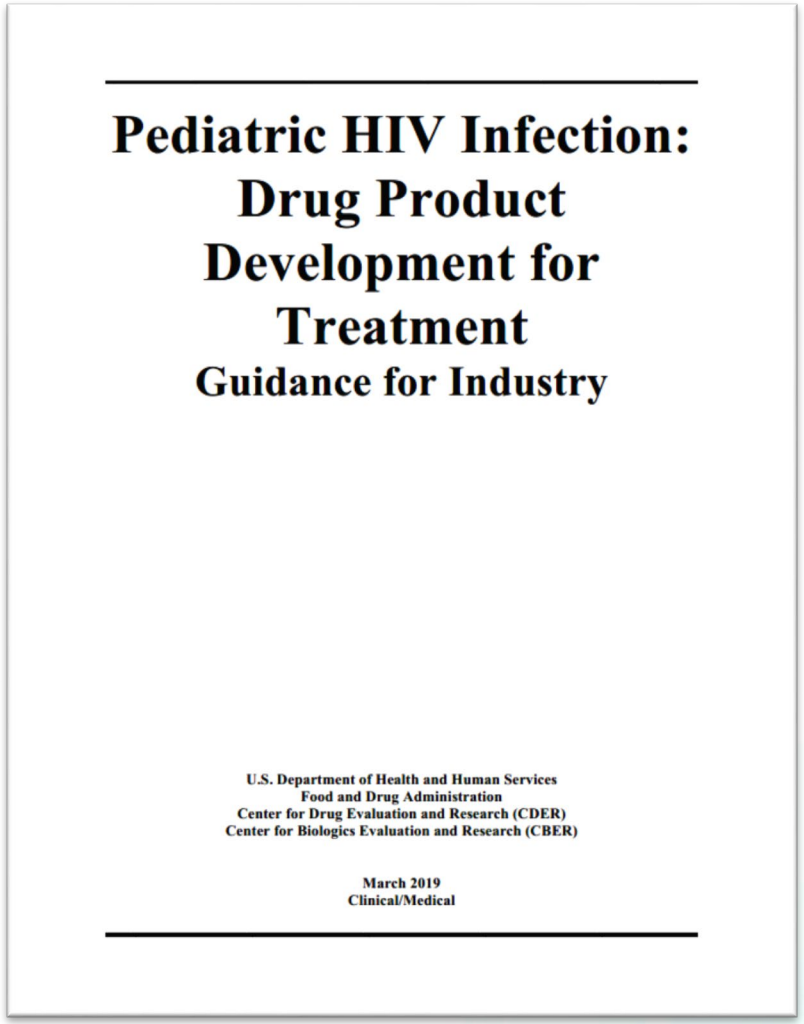
Improvements in Pediatric Approval Lag Times, but…



Lessons Learned from HIV

- Research on pediatric HIV has many advantages over other diseases that impact children
  - Extensive natural history that is similar to how it affects adults
  - Expansive research networks
  - Several classes of drugs
- Existing regulatory mechanisms are available to speed up the development and approval process

Lessons Learned from HIV



Pediatric HIV Infection: Drug Product Development for Treatment

Guidance for Industry

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

March 2019
Clinical/Medical

- New guidance for industry on pediatric HIV product development released in 2019
- Clear articulation to address questions from industry that may have been delaying research and/or product filings
- Getting the right groups to the table can lead to incremental but important changes
- FDA has an important role to play in accelerating progress
  - Formal processes like end Phase 1 and 2 meetings
  - Informal processes like meetings with advocates and industry

Related Challenges

- Lack of PREA application to orphan drugs impacts numerous pediatric diseases and conditions, including common comorbidities affecting children living with HIV
  - Tuberculosis, Hepatitis C, Malaria
- Recent report from FDA reinforces the need to apply PREA to orphan drugs
- Labeling for children for bedaqualine, the first tuberculosis medicine from a new drug class in 50 years, is not expected until **13 years** after adult approval

Conclusion

- FDA needs to use existing opportunities through end of phase 1 and end of phase 2 meetings to improve lag times in labeling new medicines for children
- PREA should be applied to orphan drugs
- Lessons of therapeutic HIV advances should be applied to other pediatric diseases
- Even with supportive policies, children access to many treatments remains suboptimal – vigilance remains necessary to improve options for them across all diseases and conditions

Elizabeth Glaser Pediatric AIDS Foundation

Until no child has AIDS.