RACE for Children Act

- Incorporated as Title V Sec. 504 of the **FDA Reauthorization Act (FDARA)**
  - Enacted August 18, 2017
  - Amended Pediatric Research Equity Act **PREA** (Sec. 505B of the FD&C Act)
- **Requires** evaluation of new molecularly targeted drugs and biologics "intended for the treatment of adult cancers and directed at a **molecular target** substantially relevant to the growth or progression of a pediatric cancer"
  - Applies to **original marketing applications** submitted on or after August 18, 2020
- **Molecularly targeted pediatric cancer investigation**: clinically meaningful study data, "using appropriate formulations, regarding **dosing, safety and preliminary efficacy** to inform potential pediatric labeling."*
- Elimination of **orphan exemption for pediatric studies** for cancer drugs directed at relevant molecular targets.

*FDARA Title V Sec 504 (a)(3)(A) or FD&C Act Sec. 505B (a)(3)(A)]

OCE Pediatric Oncology Program
RACE Act Implementation

- Development and Maintenance of Pediatric Molecular Target Lists to **guide** decision-making regarding early pediatric evaluation and iPSP submission
  - Multi-stakeholder input on target relevance obtained through public meetings

- December 2019 issuance of FDA Guidance on FDARA Implementation https://www.fda.gov/media/133440/download

- Sec. 503 Early Advice Meetings for sponsors
  - To clarify iPSP requirements for original NDAs/BLAs
  - Scheduled and held within 30 days of request

- Participation in monthly Pediatric Cluster calls and international multi-stakeholder meetings (like the ACCELERATE Strategy Forums) to facilitate global coordination and harmonization in pediatric oncology drug development

NCI Programs for Pediatric Preclinical Testing

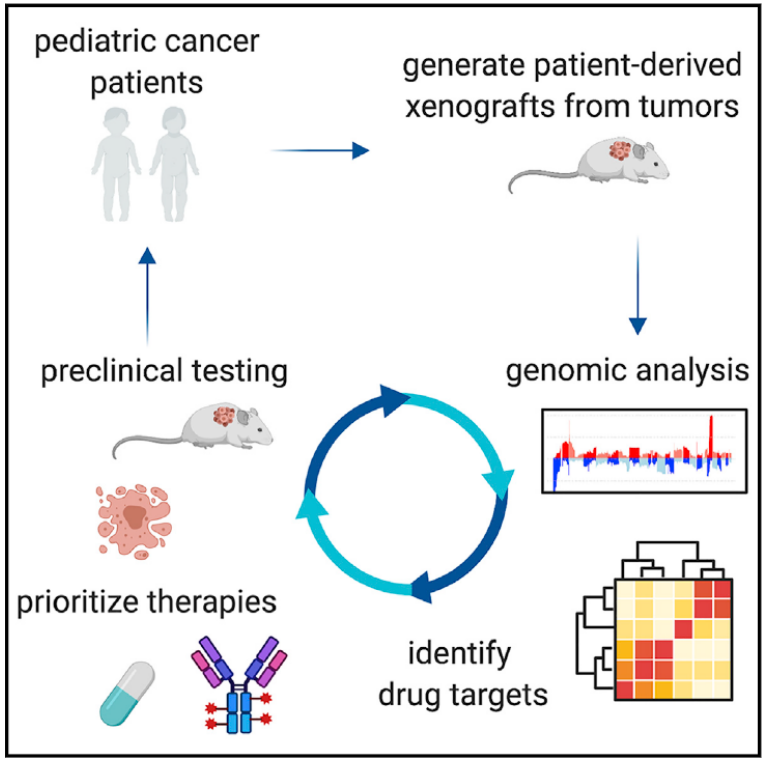
Malcolm A. Smith, MD, PhD

NIH NATIONAL CANCER INSTITUTE

January 17, 2020

NCI Pediatric Preclinical Testing

- Enormous opportunities and challenges created by RACE Act
- Preclinical testing can play central role in addressing these challenges and opportunities
- NCI has supported systematic approach to pediatric preclinical testing for more than 15 years



Rokita JL, et. al; Cell Reports 2019

Enhancing Pediatric Preclinical Testing – 2021 and Beyond

- Increase research programs involved in preclinical testing program
  - Expand numbers of models for each disease type
  - Expand to new disease types
  - Expand to new types of treatment (immuno-oncology)
  - Expand to new types of testing
- Increase testing throughput
- Increase capabilities for data sharing through a Preclinical Data Commons
- Increase opportunities for collaboration and communication with pharmaceutical companies and regulatory agencies through the FNIH public-private partnership

Pediatric Cancer Preclinical Testing Partnership

Foundation for the National Institutes of Health

FNIH
Foundation for the
National Institutes of Health

Responding to New Regulatory Requirements

- Compliance with FDARA Title V, Sec. 504 is the compelling 'impending event,' but a comprehensive approach to the public health challenges of providing robust, scientifically valid preclinical testing is the goal
- FNIH was approached to develop a Public-Private Partnership (PPP) for a centralized resource for pediatric preclinical testing
- Initial support for PPP Design Phase provided by PhRMA
- PPP was designed with representatives from 16 companies, NCI, FDA, and patient advocacy organizations using a well-tested, planning methodology used to establish other PPPs such as AMP and PACT
- PPP proposes to involve both NIH (NCI) funding as well as private sector funding
- PPP recognizes the need for a global solution and will complement established initiatives such as the IMI2 ITCC-P4 and ACCELERATE efforts in the EU

FNIH

The Design Team agreed on a joint research plan with eight key components

1. Create, publish, and continuously update a catalogue of existing preclinical pediatric cancer models and facilitate research community access to these models

2. Establish and validate a high-throughput in vitro testing platform (e.g., cell lines and potentially organoids as well), and make it broadly available to stakeholders

3. Harmonize existing standards across models, data, response criteria, and informed consent

4. Develop new murine models through collaborative pilot studies to address the key current gaps in the field,

5. Invest in enhanced and expanded “data commons” capabilities to enable aggregation and comparison of existing and new testing data to support enhanced decision-making

FNIH

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The Design Team agreed on a joint research plan with eight key components (cont'd)

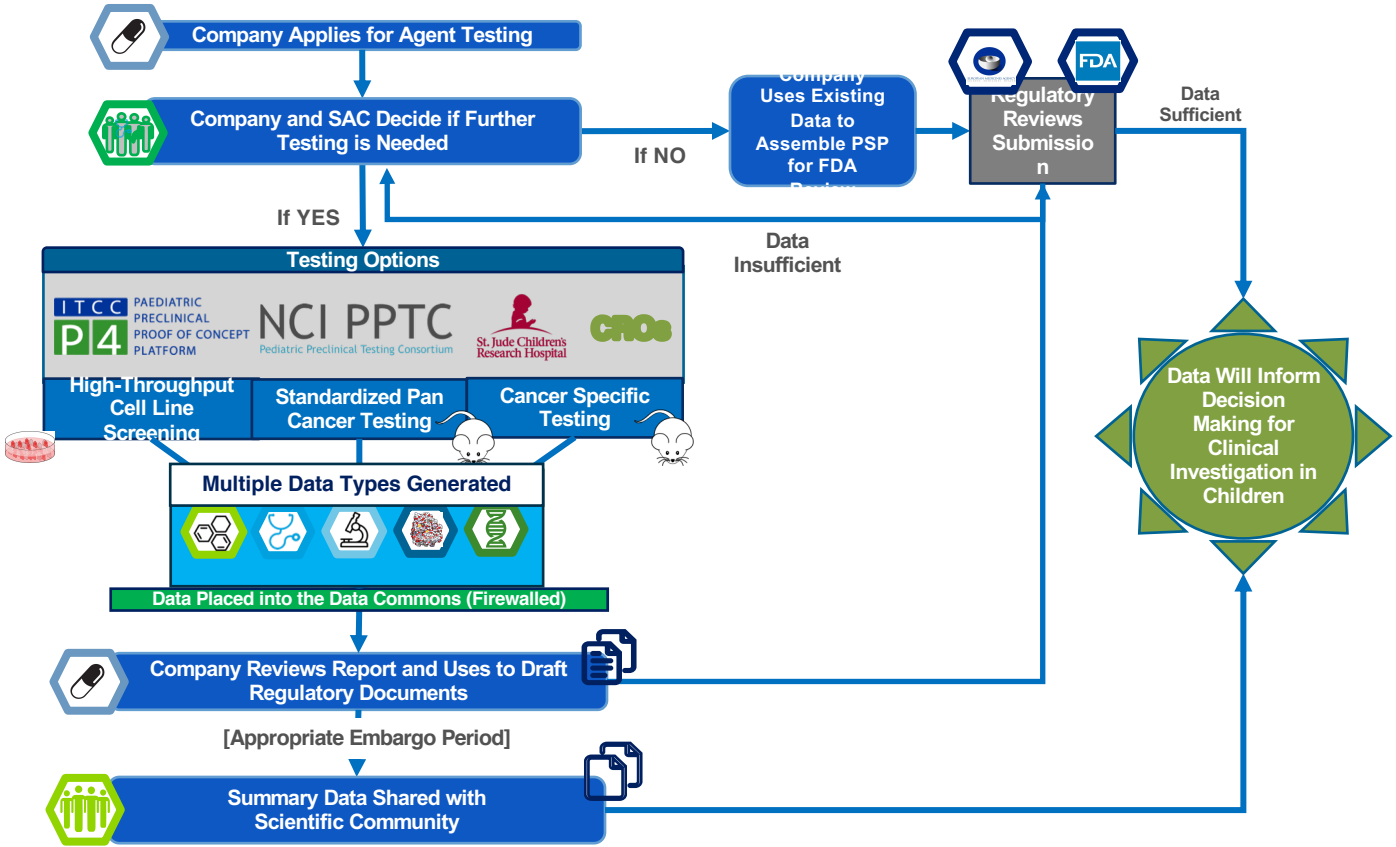
6. Establish an ongoing forum for tracking progress and allocating new investments, the Strategic Advisory Committee (SAC)

7. Conduct joint target and agent feasibility analyses – encompassing data from publicly available repositories that is unpublished as well as the data generated by the testing conducted within the PPP

8. At company option, conduct testing of agents at sites established by the PPP – either CRO or academic sites. Make results available to the broader research community (once appropriate exclusivity periods are met)

FNIH

PPP seeks to unify existing testing efforts, aggregate data, and make broadly available to the community



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