Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee: Introductory remarks

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US Food and Drug Administration

ODAC Agenda

Aim: to discuss perspectives relating to implementation of 2017 FDARA legislation and its impact on pediatric cancer drug development to date

- Presentations from regulators (FDA and EMA)
- U.S. cooperative group perspectives
- Industry perspectives
- Perspectives on international trial collaboration
- Clarifying questions and discussion

FDARA: Food and Drug Reauthorization Act; EMA: European Medicines Agency

Elements of iPSP development

Sponsor iPSP preparation

- Preclinical proof of concept studies in pediatric preclinical investigations
- Stakeholder interaction encouraged to inform patient selection, study feasibility, and design

FDA decision-making

- Scientific support for proposed pediatric investigation (e.g., applicable adult clinical and nonclinical data, nonclinical proof of concept data in relevant pediatric models)
- Independent assessment of potential for benefit of targeted drug in pediatric patients with cancer
- Consideration of pediatric development landscape

Collaboration on iPSPs occurs within FDA and with stakeholders

Global, multistakeholder opportunities for collaboration on pediatric development plans due to the RACE Act

International regulators ⟷ Investigators/Sponsors ⟷ Patient advocates

- Timelines for iPSP and PIP (EMA) more closely aligned
- Pediatric Cluster Call and Common Commentary
- EMA participation in Pediatric Subcommittee of ODAC meetings

- FNIH/COACH meetings
- ACCELERATE pediatric strategy forums
- Participation in Pediatric Subcommittee of ODAC meetings

- FDA listening sessions
- OCE Project community
- ACCELERATE pediatric strategy forums
- Participation in Pediatric Subcommittee of ODAC meetings

Discussions and input from all stakeholders are vital to optimize use of existing resources and promote efficient timely development of new drugs for pediatric patients with cancer

PIP: Paediatric Investigation Plan; EMA: European Medicines Agency; FNIH: Foundation for the National Institutes of Health; COACH: Convening Experts in Oncology to Address Children's Health; OCE: Oncology Center of Excellence

Expectations and discussion

- Perspectives on how FDARA is impacting pediatric oncology ecosystem
- Considerations for preclinical proof-of-concept studies in pediatrics
- Discussion on the role of international collaboration in efficient development of new therapies for pediatric patients with cancer

Effective and **consistent** communication among stakeholders is critical to optimize decision-making


FDA
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ADMINISTRATION

FDA Reauthorization Act (FDARA) Amendments to the Pediatric Research Equity Act: FDA Perspectives and Updates on Implementation

Meeting of the Pediatric Oncology Subcommittee
of the Oncologic Drugs Advisory Committee
May 22, 2024

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Office of Oncologic Diseases
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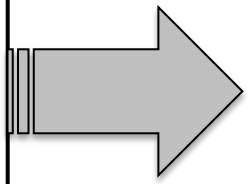
Outline

- Legislative and regulatory landscape
- Products subject to FDARA requirements
  - Molecular targets and target relevance
  - Application of FDARA provisions of PREA
  - Considerations for cell and gene therapy (CGT) products
- FDARA Implementation
  - Scope of pediatric investigations and grounds for waivers and deferrals
  - Content of iPSPs and approach to regulatory decision-making
  - International multistakeholder collaboration
- Early measures of FDARA impact and future steps

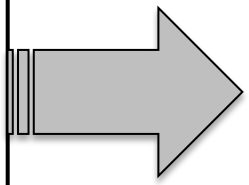
Timeline of key FDA legislation impacting pediatric drug development

1902 Biologics Control Act
1906 Pure Food and Drug Act
1938 Food Drug and Cosmetic Act
1962 Kefauver-Harris Amendment



Early legislation reflected a response to products that caused harm

1979 *Pediatric Use* subsection of USPI
1997 FDAMA/Pediatric exclusivity provision
2002 Best Pharmaceuticals for Children Act (BPCA)
2003 Pediatric Research Equity Act (PREA)
2017 FDA Reauthorization Act (FDARA)



Later pediatric legislation encourages or requires pediatric investigations to inform product labeling

Legislation to incentivize or require pediatric drug development

Voluntary	Mandatory
• **BPCA (2002) → PPSR/WR** – Plan for entire pediatric development program – Intended to support labeling claims / expanded indication – Once fulfilled, provides for 6-months of pediatric exclusivity	• **PREA (2003) → iPSP**

PPSR: proposed pediatric study request
WR: written request
iPSP: initial pediatric study plan

Legislation to incentivize or require pediatric drug development

Voluntary	Mandatory
• **BPCA (2002) → PPSR/WR** – Plan for entire pediatric development program – Intended to support labeling claims / expanded indication – Once fulfilled, provides for 6-months of pediatric exclusivity	• **PREA (2003) → iPSP** – Early in development – Outline of planned pediatric study/studies – May contain plan to request waiver, partial waiver, or deferral

PPSR: proposed pediatric study request
WR: written request
iPSP: initial pediatric study plan

Historical implications of PREA in pediatric oncology

Prior to 2020, PREA had **little or no effect in oncology** because:

- Most applications were either exempt (e.g., due to orphan drug status), or
- The pediatric study requirement was waived based on the adult indication, which did not occur in children

After 2020, FDARA amendments to PREA under the RACE for Children Act were implemented to **accelerate** the creation of a **pediatric development plan** and ultimately the development of **promising drugs for pediatric patients with cancer**

Requirements under FDARA amendments to PREA

- Reports on a molecularly targeted pediatric cancer investigation must be submitted for applications for certain drugs directed at a **molecular target** substantially relevant to the growth or progression of a pediatric cancer, unless requirement is waived or deferred

- Apply even if the drug is for an adult indication that has received orphan designation

- FDA mandated to establish, publish, and regularly update a list of molecular targets considered to be relevant and a list of non-relevant targets

2021 FDA Guidance for Industry: FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of the FD&C Act

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  - Molecular targets and target relevance
  - Application of FDARA provisions of PREA
  - Considerations for cell and gene therapy (CGT) products

- FDARA Implementation

- Early measures of FDARA impact and future steps

Which applications are subject to FDARA*?

Unless waived or deferred, reports from a molecularly targeted pediatric cancer investigation must be submitted with a marketing application if:

- ✓ The application is original
- ✓ The product is a new active ingredient
- ✓ The product that is the subject of the application is intended for treatment of an adult cancer
- ✓ **The product is directed at a molecular target** FDA determines to be substantially relevant to the growth or progression of a pediatric cancer

*criteria for applications that are subject to amendments made by FDA reauthorization act (FDARA) section 504 to section 505B of the FD&C act (also known as the Pediatric Research Equity Act, or PREA).

Molecular target lists

- The FDA maintains a publicly accessible list of *relevant* molecular targets that may trigger the requirement for pediatric investigations
  - Absence of target on the relevant list does not necessarily imply that the target is not substantially relevant

- FDA maintains a separate list of molecular targets that are considered *non-relevant* and therefore would not be subject to FDARA amendments to PREA
  - e.g., androgen receptor, estrogen receptors 1 and 2, prostate stem cell antigen, Bruton's tyrosine kinase

- FDA periodically updates both lists and encourages public comments

2021 FDA Guidance for Industry: FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of the FD&C Act

What is a molecular target?

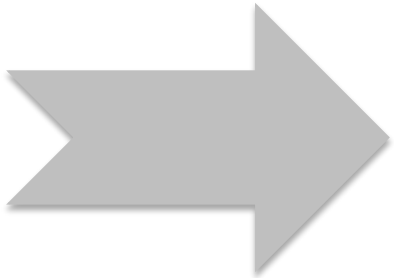
- A molecule in human cells (normal or cancer cells) that is intrinsically associated with a particular malignant disease process

- There should be evidence that **addressing (e.g., binding to, interacting with) the molecule** with a drug produces a **measurable effect on a cancer** which may translate clinically to a **favorable objective change in the disease process**

2021 FDA Guidance for Industry: FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of the FD&C Act

CDER-regulated drug and biological oncology products

- Small molecules (e.g., kinase inhibitors)
- Some biological products (e.g., monoclonal antibodies, antibody-drug conjugates, cytokines)



Molecularly targeted therapy determination is often relatively straightforward

CDER: Center for Drug Evaluation and Research

Wide range of oncology CGT products

Gene therapies (GT)

- Ex vivo genetically modified cells (e.g., CAR T Cells)
- DNA/RNA vectors (e.g., plasmids, mRNA products)
- Genome Editing Products (e.g., CRISPR)
- Replication-deficient viral vectors (e.g., Adenovirus, Adeno-associated virus, Lentivirus)
- Oncolytic Replication-competent viral vectors (e.g., Measles, Adenovirus, Vaccinia)
- Microbial vectors (e.g., Listeria, Salmonella)

Cellular Therapies

- Antigen Presenting Cell Based Therapies (e.g., DC Therapies)
- T cell Therapies (e.g., TILs)
- NK Cell Therapies
- Mesenchymal Stromal Cells (MSCs)
- Autologous/Allogenic Irradiated Live tumor Cells (e.g., GM-CSF expressing tumor cell vaccines)
- Induced pluripotent stem cells (iPSCs)
- Tissue Engineered Products (TE)

Therapeutic Vaccines

- Tumor Associated Antigen (TAA) vaccines
- Neoantigen Vaccines
- Vectored Vaccines
- Nucleic Acid Vaccines
- Peptide Vaccines
- Protein Vaccines
- Cellular Vaccines

Complexity of CGT products

Determining which CGT Products are molecularly targeted therapies (MTT) can be complex because:

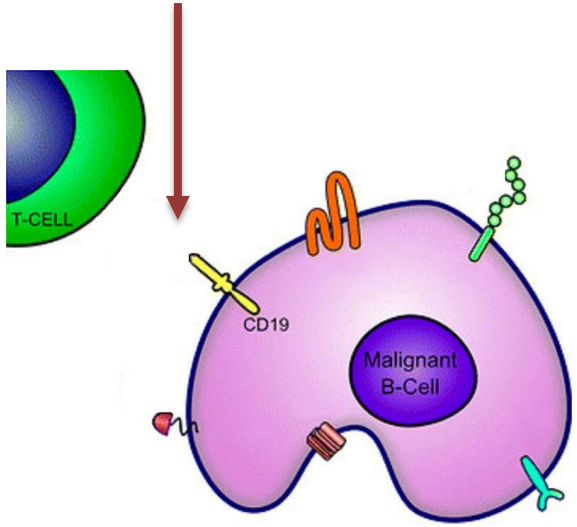
- Molecular basis for the mode of action (MOA) may not always be associated with well characterized or recognizable targets
- A specific CGT product can have multiple MOAs
- Role of interaction between a given target and tumor cell may differ between small molecule or antibody-based therapies and CGT products

Thus, a one size-fits-all approach may not be feasible and decisions regarding which CGT products are MTT are often made on a case-by-case basis

Example of MTT determinations

CAR T-Cell Therapy

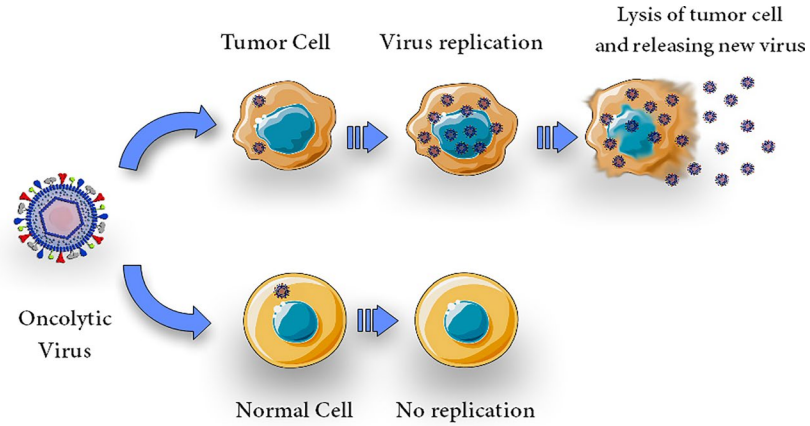
Interaction with CD19 target is integral to anticancer MOA



CAR T Cells: Engineering Immune Cells to Treat Cancer - NCI

Oncolytic therapy

MOA for tumor cell lysis may not rely on interaction with one or more specific molecules on either cancer or normal cells



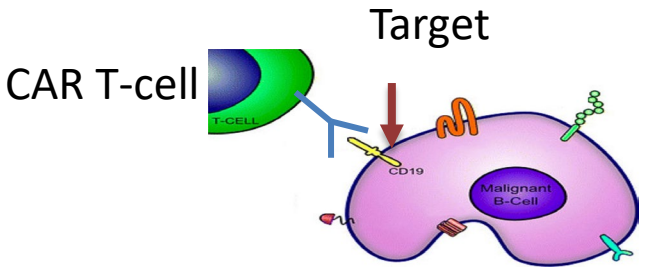
Front. Immunol., Cancer Immunity and Immunotherapy Volume 13 - 2022
https://doi.org/10.3389/fimmu.2022.1012806

CGT products generally considered MTT

- ✓ CAR T Cells
- ✓ Genome Edited Cells
- ✓ Cancer Vaccines against specific targets (TAAs/viral antigens/other known targets)
- ✓ T Cells directed at specified target antigens (e.g., TAAs)

Product interacts with one or more specific molecules associated with cancer or normal cells

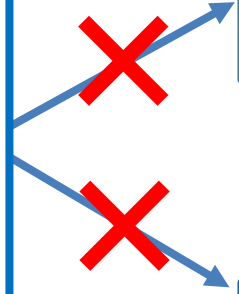
Interaction with the molecule(s) generally correlates with activity and tumor killing (interacting with the molecule(s) can produce a measurable effect)



TAA: Tumor Associated Antigens (e.g., P53, NY-ESO1, CEA)

CGT products generally not considered MTT

- ✓ Mesenchymal Stem Cells (MSCs)
- ✓ Induced Pluripotent Stem Cells (IPSCs)
- ✓ Tissue Engineered Products (TE)
- ✓ Patient Specific Neoantigen Vaccines



Product interacts with one or more specific molecules associated with cancer or normal cells

Interaction with the molecule(s) generally correlates with activity and tumor killing (interacting with the molecule(s) can produce a measurable effect)

Neoantigens: Patient-specific mutated cancer cell proteins (or epitopes) that are identified using High Throughput Sequencing (HTS) and Bioinformatic algorithm-based analysis

Are Neoantigen- and TAA-based cancer vaccines MTTs?

TAA vaccine
Not patient-specific
Generally considered MTT

Yes

Neoantigen vaccine
Patient-specific
Generally not considered MTT

No

Product interacts *(through immune cells)* with one or more specific molecules associated with cancer or normal cells

AND

Interaction *(of the antigen stimulated immune cells)* with the *(specified)* molecule(s) *(on cancer cells)* generally correlates with activity and tumor killing (interacting with the molecule(s) can produce a measurable effect)

TAA: Tumor Associated Antigens

CGT products which may or may not be considered MTT

- ✓ **Oncolytics:** replication of competent viruses and bacteria that infect tumor cells and lyse them: e.g., Adenovirus; Pox Virus; Clostridium bacteria
- ✓ **Neoantigen specific T cells:** T cells expanded in the presence of neoantigens
- ✓ **TILs:** Tumor infiltrating lymphocytes

Key takeaways

- Determining which CGTs are MTT is not always straightforward
  - CGTs may work through MOA that is independent of specific targets on tumor cells
  - MOA may vary depending on the manufacturing process
  - When the MOA is not dependent on specified targets on cancer cells, MTT determinations are made on a case-by-case basis
- Early interactions with FDA can help sponsors prepare iPSPs for CGTs

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- Products subject to FDARA requirements
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  - Scope of pediatric investigations and grounds for waivers and deferrals
  - Content of iPSPs and approach to regulatory decision-making
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Scope of molecularly targeted pediatric cancer investigations

- Typically, non-hypothesis testing single arm trials evaluating single agents or combinations
- Objectives include:
  - Evaluation of tolerability and identification of DLTs
  - Evaluation of PK across relevant age groups
  - Identification of RP2D and schedule
  - Preliminary assessment of activity (e.g., ORR and DOR) in overall population and pertinent subsets (e.g., based on biomarker enrichment of tumor type)

If required study shows sufficient evidence of antitumor activity, the FDA may consider issuing a Pediatric Written Request for more definitive evaluation

DLT: dose-limiting toxicity; PK: pharmacokinetics;
RP2D: recommended phase 2 dose;
ORR: overall response rate; DOR: duration of response

Statutory grounds for deferral of molecularly targeted pediatric investigations

FDA may agree to defer submission of some or all reports if:

- Drug is ready for approval for use in adults before pediatric investigation(s) are complete
- Pediatric investigation should be delayed pending availability of additional data
  - e.g., data from proof of concept studies or additional clinical data
- There is another appropriate reason

Sponsors must provide plan for timely initiation of studies and evidence that they are being conducted with due diligence as early as possible

Section 505B(a)(3)(B)(i)-(iii) of the Food, Drug and Cosmetic Act)

Statutory grounds for a full or partial waiver

- Necessary studies impossible or highly impracticable (e.g., due to rarity of population)
- Evidence strongly suggests the drug/biologic would be ineffective or unsafe in all or certain pediatric age groups (for partial waiver)
- Drug does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients AND is not likely to be used by a substantial number of pediatric patients
- Reasonable attempts to produce an appropriate pediatric formulation have failed (for partial waiver)

Section 505B(a)(4)(A) of the Food , Drug, and Cosmetic Act

Same-in-class products:
FDA considerations for agreement with plans for waiver

- ✓ No evidence product would provide superior pharmacologic, safety, or antitumor activity compared to other drugs in the same class

and

- ✓ Ongoing or competing studies in pediatric patients underway and additional studies not feasible due to small patient numbers

or

- ✓ One or more drugs in the same class failed to show activity that would warrant additional studies

Examples: PD-1/PD-L1 axis inhibitors, PI3K delta isoform inhibitors, EGFR inhibitors, FGFR inhibitors, CD20-directed antibodies

2021 FDA Guidance for Industry: FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of the FD&C Act

2022 Pediatric Oncology Subcommittee of ODAC Meeting

Considerations for same-in-class decision-making

Members opined that the following factors are important:

- Comparative clinical and nonclinical data (toxicity and efficacy)
  - adults (clinical and nonclinical)
  - pediatric nonclinical models
  - overlapping biology between adult and pediatric cancers (in some cases)
- Cancer rarity and feasibility of investigations
- Potential for efficacy in pediatrics
- Toxicity profile
- Unmet need/disease prognosis
- Others (dosage form, route of administration, dosing frequency, drug-drug interactions, palatability, combination potential, CNS penetration)

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Sponsor iPSP preparation

Early planning and stakeholder engagement are important to facilitate timely iPSP development and inform FDA decision-making

- Nonclinical proof of concept studies in relevant pediatric models encouraged early during product development:
  - Identification of pediatric population(s) most likely to derive benefit
  - Resources include NCI-supported PIVOT and ITCC-P4 programs

- Type F meetings for iPSP planning (for CDER products)
  - Inclusion of European Medicines Agency (EMA), investigators, and patient advocate representatives is encouraged
- Stakeholder interaction strongly encouraged to inform patient selection, study feasibility and design

PIVOT: Pediatric Preclinical In Vivo Testing
ITCC-P4: Innovative Therapies for Children Paediatric Proof-of-Concept

iPSP content informing FDA decision-making

Important information provided by sponsors to support the proposed plan:

- Systematic review of available evidence supporting target relevance to pediatric cancers (e.g., through public genomic databases, literature)
  - Estimate of target prevalence in pediatric cancers
  - Role of target in growth or progression of pediatric cancers
- Clinical and nonclinical data in adults
- Available proof of concept information in relevant pediatric cell lines and in vivo models
- Landscape of development pertinent to drug class or proposed pediatric patient population
- Summary of stakeholder perspectives

FDA iPSP review is collaborative

- OCE Subcommittee of the PeRC meetings:
  - Include oncology and general pediatrics, clinical pharmacology, genomics, nonclinical pharmacology/toxicology, legal, ethics, and regulatory experts
- Comments and requests for information issued to sponsor
- Discussion occurs with representatives of the EMA during cluster calls if requested by the sponsor, or at the initiative of either agency
- In communication with the sponsor, FDA can amend Agreed iPSPs based on evolving scientific, nonclinical, and clinical information

OCE: Oncology Center for Excellence;
PeRC: Pediatric Review Committee;
EMA: European Medicines Agency

Outline

- Legislative and regulatory landscape

- FDARA Implementation
  - Scope of pediatric investigations
  - Waivers and deferrals
  - Content of iPSPs and approach to regulatory decision-making
  - International multistakeholder collaboration

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International multistakeholder collaboration is vital

Number of targeted drugs >> number of pediatric patients available for clinical trials

Evolving scientific understanding of pathophysiology of pediatric cancers

Global coordination of all stakeholders including advocates is necessary to:

- Prioritize drugs of interest for early pediatric evaluation
- Develop strategies for drug development for specific rare cancers

Prevents duplication of studies and competition for patients

Limits unnecessary exposure of pediatric patients to investigational drugs

EMA and FDA scientific review of pediatric cancer study plans

- With FDARA, timelines for iPSP and PIP (EMA) more closely aligned
  - New PIPs and iPSPs should be submitted simultaneously
  - Pediatric Cluster Call and Common Commentary process provide forum for preliminary scientific advice from both the EMA and the FDA on PIPs and iPSPs
- EMA invited to observe FDA Type F meetings with sponsor permission
- EMA participation in FDA mini-symposia and Pediatric Subcommittee of ODAC meetings
- FDA and EMA included in pediatric oncology-related discussions coordinated by the respective agency, with sponsor permission

PIP: Paediatric Investigation Plan

Other multistakeholder efforts

- ACCELERATE Pediatric Strategy Forums
  - May 2024 meeting on diffuse midline glioma
  - October 2023 meeting on CDK 4/6 and PI3K inhibitors
- FDA listening sessions with patient advocates and representatives
- OCE Project Community
- FDA mini-symposia with external constituents
  - May 2023 meeting on challenges in trial design for relapsed/refractory osteosarcoma
  - October 2022 meeting on functional outcomes as efficacy endpoints in LGG
  - February 2022 meeting on pediatric enrollment in cancer clinical CGT trials
- Foundation for the National Institutes of Health (FNIH) Convening Experts in Oncology to Address Children's Health (COACH) meeting discussions

Discussions and input from all stakeholders are vital to optimize use of existing resources and promote efficient timely development of new drugs for pediatric patients with cancer

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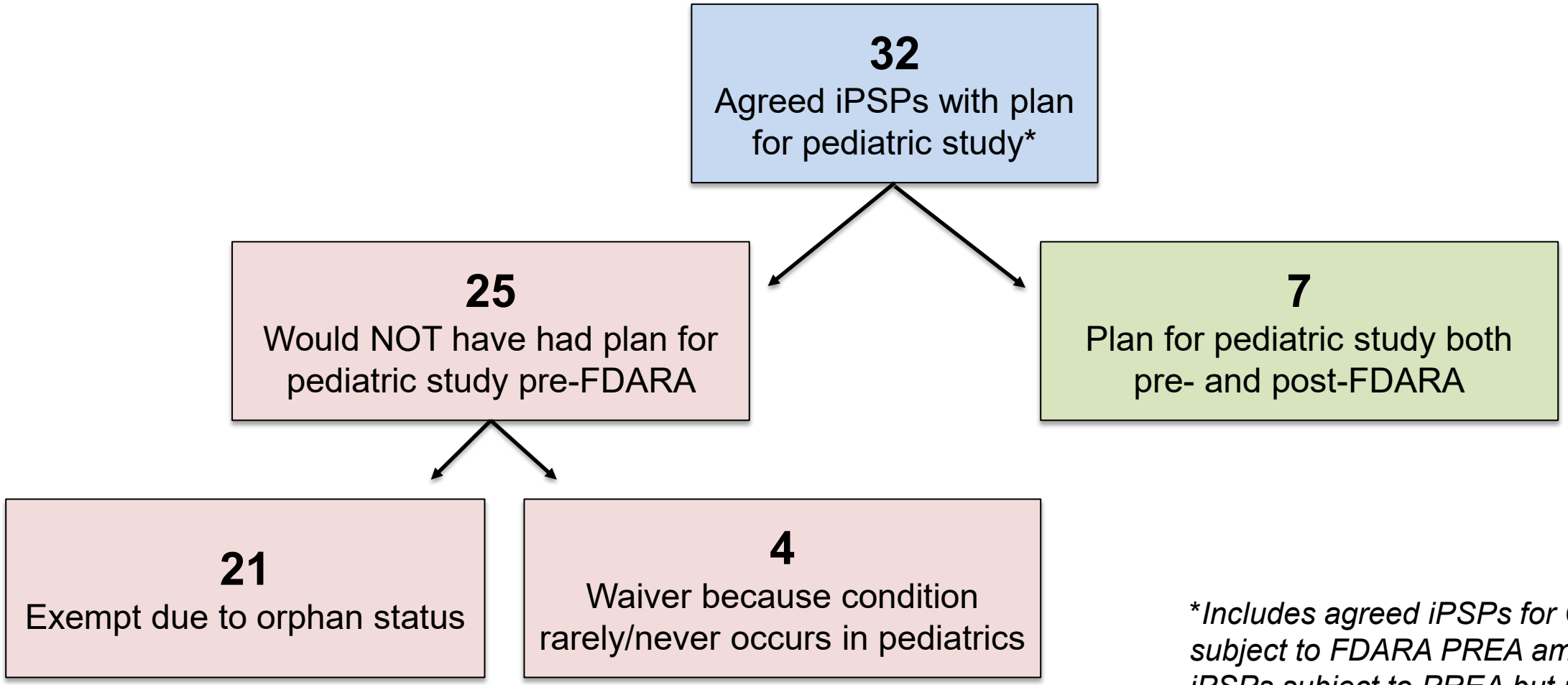
Early impact of FDARA

Agreed iPSPs with Planned Pediatric Studies
August 18, 2020, through August 18, 2022



**Includes agreed iPSPs for CDER products subject to FDARA PREA amendments and iPSPs subject to PREA but not FDARA amendments to PREA*

Early impact of FDARA

Agreed iPSPs for investigational new drugs directed at a substantially relevant molecular target
August 18, 2020, through April 18, 2024

Agreed upon plan	Agreed iPSP* N=96
Planned request for full waiver n (%)	42 (44%)
Planned molecularly targeted pediatric cancer investigation n (%)*	54 (56%)

*The majority (~80%) of the 54 agreed upon plans to conduct a molecularly targeted pediatric cancer investigation include a plan for partial waiver, deferral, or both

*CDER products

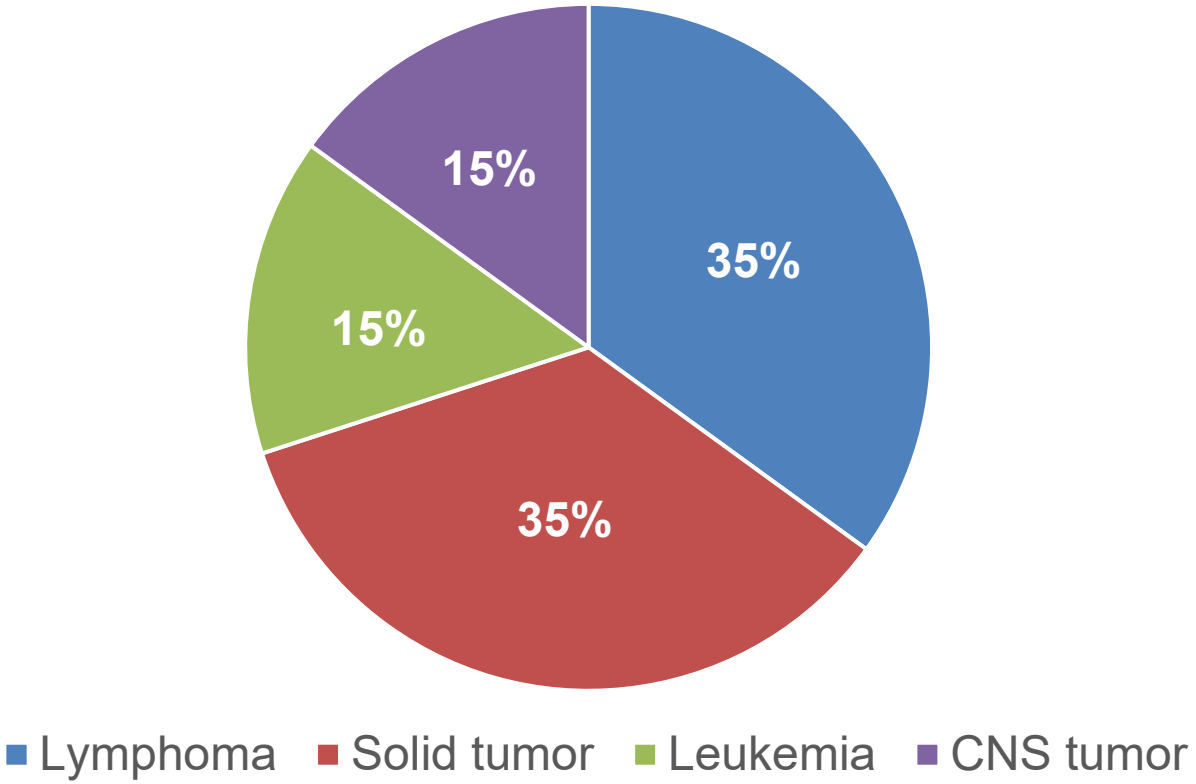
Early impact of FDARA

Pediatric studies required under PREA
August 18, 2020, through April 18, 2024

Targeted drugs with PREA post-marketing requirement	**N=17 n (%)**
Application for new active ingredient subject to FDARA PREA provisions	14 (82%)
Application for previously approved drug not subject to FDARA PREA provisions	3 (18%)
Clinical studies deferred pending additional proof-of-concept data	3 (18%)
Clinical studies with partial waiver for one or more pediatric age groups	11 (65%)

PMRs issued for pediatric studies under PREA August 18, 2020, through April 18, 2024

Pediatric cancer type



Molecular target

CD19	BCR-ABL1
CTLA4	IDH1
PD-1, LAG3	BRAF V600
FLT3	HER2
FGFR	AKT
CD20-CD3*	ALK, ROS1, NTRK

**Bispecific antibody*

Early measures of FDARA impact: Summary and future steps

- Based on FDA and GAO analyses, **FDARA has increased the development of targeted therapies in pediatric patients since its implementation in August 2020** by increasing
  - Number of agreed iPSPs with plans for pediatric investigations
  - Number of post-marketing requirements for pediatric studies

- Given timelines needed for initial pediatric investigation and definitive trials, it is too early to determine whether FDARA legislation will result in more approvals of new drugs for pediatric patients with cancer

- FDA is committed to monitoring progress of pediatric investigations required under FDARA

Improving the positive impact of FDARA implementation

- Early and frequent engagement among all stakeholders and international collaboration is crucial to:
  - Prioritize agent investigation and development
  - Assess research strategy
  - Harmonize goals and increase efficiency

Decision-making should be continually reassessed to refine implementation of pediatric regulations under BPCA and PREA to maximize the benefit to pediatric patients with cancer

Important resources

- Oncology Center of Excellence Pediatric Oncology Program: https://www.fda.gov/about-fda/oncology-center-excellence/pediatric-oncology
- Pediatric oncology drug approvals: https://www.fda.gov/about-fda/oncology-center-excellence/pediatric-oncology-drug-approvals
- Relevant molecular target list: https://www.fda.gov/media/161463/download?attachment
- Non-relevant molecular target list: https://www.fda.gov/media/161462/download?attachment
- Public docket for input on molecular target lists: https://www.regulations.gov/document/FDA-2018-N-3633-0001
- Guidance on FDARA Implementation: https://www.fda.gov/media/133440/download
- Considerations for the Inclusion of Adolescent Patients: https://www.regulations.gov/docket/FDA-2018-D-1540
- Best Pharmaceuticals for Children Act: https://www.fda.gov/drugs/development-resources/best-pharmaceuticals-children-act-bpca
- Written Requests Issued: https://www.fda.gov/drugs/development-resources/written-requests-issued
- Guidance on Pediatric Study Plans: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/pediatric-study-plans-content-and-process-submitting-initial-pediatric-study-plans-and-amended

CBER Contact Information

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

  http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm

FDA Headquarters

- **CBER website:** www.fda.gov/BiologicsBloodVaccines/default.htm
- **Phone:** 1-800-835-4709 or 240-402-8010
- **Consumer Affairs Branch**: ocod@fda.hhs.gov
- **Manufacturers Assistance and Technical Training Branch**: industry.biologics@fda.gov
- **Follow us on X, formerly Twitter:** https://www.twitter.com/fdacber

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- Sarah Zaidi
- DACCM Staff
- OCE Subcommittee of the PeRC members
- All OCE and OOD staff

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