INC
INTERNATIONAL NEONATAL CONSORTIUM

Cell and Gene Therapy: RWE Workgroup

Project Overview: Mapping Global Registries for Cell and Gene Therapy (CGT) Development in Neonates and Infants

OBJECTIVE

The Cell and Gene Therapy (CGT) Real-World Evidence (RWE) Workgroup, part of the International Neonatal Consortium (INC), is focused on advancing the development of CGT for neonates and infants. The project aims to map existing patient registries that support CGT development globally for infants and provide recommendations for key data elements needed in these registries. This effort will contribute to improved regulatory alignment, patient recruitment, and potential for external controls for CGT clinical trials.

PROJECT DELIVERABLES

1. **White Paper:**

The final white paper will include:

- **Survey Results:** Overview of patient registries focusing on rare diseases, infants, and CGT.
- **RWE Use Cases:** Discussion of RWE use for patient recruitment and external control in CGT trials involving neonates/infants.
- **Core Elements for Registries**: Recommendations for essential data elements for patient registries that include infants with the potential need for CGT.
- **Standards & Regulatory Alignment:** Exploration of RWD/RWE standards and their alignment with gene therapy trials and regulatory requirements.

2. **Recommendations**:

Core data elements for patient registries that include infants with potential for CGT.

PROJECT ACTIVITES

- **Survey Development:**

A comprehensive survey will be developed to gather information about global registries supporting CGT development for neonates/infants. Key areas of focus include:

  - **Registry Type:** Population-based, active recruitment, voluntary sign-up.
  - **Registry Host:** Academic research groups, patient organizations, hospitals/healthcare systems.
  - **Indications/Mutations Covered:** Targeted diseases and genetic conditions.
  - **Genetic and Intervention Data:** Inclusion of genetic data, fetal intervention data.
  - **Accessibility:** Accessibility for both academia and industry.
  - **Data Standards:** Identification of core datasets, data dictionaries, and data standards used.
  - **Fit for Regulatory Submissions:** Suitability for regulatory submission purposes.

- **Global Registry Identification:**

Identify and prioritize rare disease patient registries globally that include infants and are relevant for CGT development.

- **Survey Analysis and Consensus Building:**

After survey data collection, a multistakeholder consensus will be built to define core data elements for registries involving infants with CGT potential.

- **White Paper Authorship:**

A comprehensive white paper will be authored, presenting survey findings, RWE use cases, and recommendations for standardization.

TIMELINE (1 YEAR)

NOV. 2024 - MAY 2025

- Develop and distribute patient registry survey.
- Prioritize registries relevant to rare diseases in infants with active CGT pipelines.
- Conduct the survey and collect data.

JUNE 2025 - DEC. 2025

- Analyze survey results and obtain multistakeholder feedback.
- Author and finalize the white paper.

CONCLUSION

This project aims to enhance global understanding of the landscape of patient registries supporting Cell and Gene Therapy development for neonates and infants. By identifying best practices, establishing data standards, and fostering collaboration, the INC's Cell and Gene Therapy RWE Workgroup seeks to accelerate the development of life-changing treatments for this vulnerable population.

For more information, visit C-Path.org.

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