



Emerging Technologies and Innovation in Manufacturing Regenerative Therapies: A Workshop

The Rare Disease Patient Perspective

Karin Hoelzer, DVM, PhD

Director, Policy and Regulatory Affairs

NORD® MISSION STATEMENT

We improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.

REGENERATIVE MEDICINE IS OFTEN THE BEST (AND ONLY) HOPE FOR OUR PATIENTS

It's estimated that

25-30

MILLION AMERICANS

(almost 1 in 10) have rare diseases.⁷

More than

90%

of rare diseases are **WITHOUT** an FDA-approved treatment.⁹



Many rare diseases result in premature deaths of infants and young children, or are fatal in early childhood.⁴

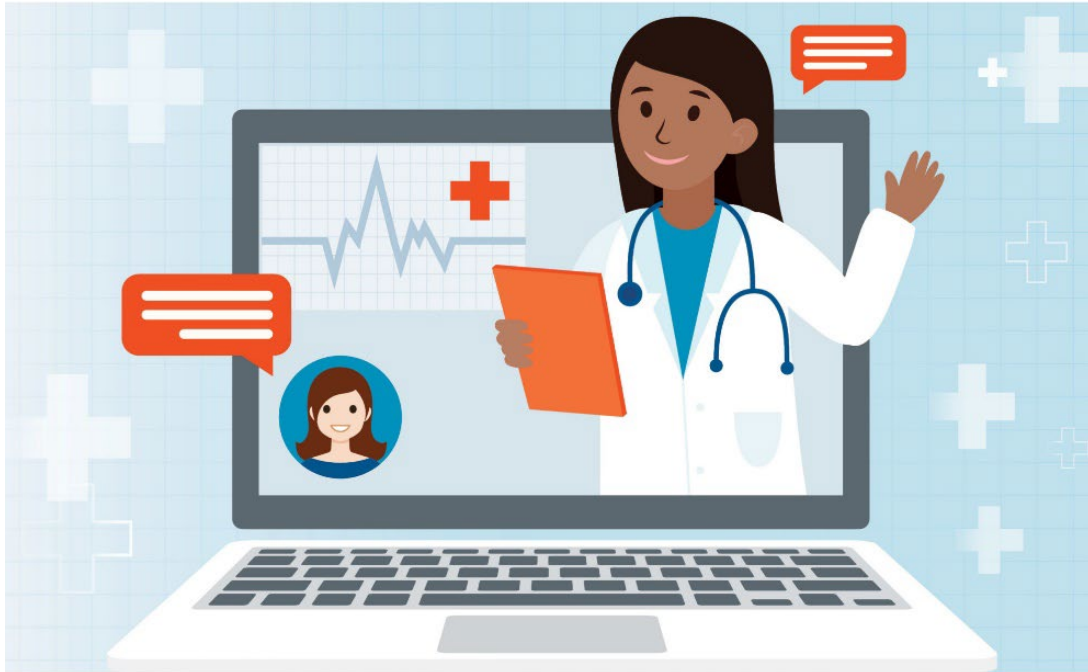


Most rare diseases are **genetic** or have a genetic component.³



- Most rare disease patients have **NO FDA-APPROVED TREATMENTS** and **NO CURES**
- Many rare diseases have a **GENETIC** component and lead to **PREMATURE DEATH** in **CHILDHOOD**
- Most rare diseases have heterogenous **DISEASE MANIFESTATIONS** and **PROGRESSIONS**
- Most cell & gene therapies developed to date have been for cancer or **RARE DISEASES**
- Cell and gene therapies have completely changed the **DISEASE TRAJECTORY** for patients and families
- Future **PIPELINE** of therapies gives hope to families

MANUFACTURING IS CURRENTLY NOT A KEY CONCERN FOR OUR PATIENTS



NORD Survey: Key cell & gene therapy concerns

- Is the therapy **A CURE?** **DURABILITY** of effect?
- Why are there **VARIATIONS IN RESPONSE?**
- Is the therapy **SAFE?** What are **SIDE EFFECTS?**
- Risk for **FUTURE GENERATIONS?**
- Differences among therapy **APPROACHES?**
- Who will be able to **RECEIVE** these therapies?
-

3 KEY DETERMINANTS OF FUTURE AVAILABILITY & ACCESS FOR OUR PATIENTS



- Timely & successfully navigate **FDA REVIEW** and **APPROVAL**
 - Manufacturing clearly plays a key role
- Ensure **AFFORDABILITY** under innovative **PAYMENT MODELS**
 - Manufacturing clearly plays a key role
- Provide timely, equitable & local **ACCESS** to these therapies
 - Manufacturing clearly plays a key role

Rare disease patients are **amazing advocates**. Help patients understand why manufacturing matters so much, and which policy solutions will be effective.

THANK YOU

Karin Hoelzer, DVM, PhD

Director, Policy and Regulatory Affairs
National Organization for Rare Disorders (NORD)
Email: khoelzer@rarediseases.org
Phone: 607-339-6561

