Webinar: Equitable Access to Rare Diseases Therapies

Center for Healthcare Innovation

WEBINAR SUMMARY

Wednesday, February 23, 2021 | 11:00 AM - 12:00 PM CT

Access the webinar: https://www.chisite.org/equitable-access-to-rare-diseases-therapies

Next year marks the 40th anniversary of the Orphan Drug Act, which established incentives to research and develop indications for rare diseases. Since then, 1,039 "orphan indications" have been approved by the FDA for previously unaddressed medical conditions, with 26 approved in just 2021 alone. Advancing technologies have enabled healthcare providers to diagnose rare conditions that impact 1 in 10 Americans more effectively - thanks mainly to advocacy and legislative efforts to expand and fund new-born screening. The general public has also become aware of and increasingly familiar with rare diseases as new indications therapies have received marketing authorization from the FDA. In this webinar, distinguished panelists representing diverse patient communities, healthcare providers, multifaceted manufacturers, and patient support providers will address opportunities and challenges to equitable access to rare disease therapies.

50% *Black* Americans

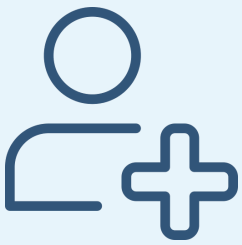
42% *Native* Americans

34% *Asian* Americans

33% *Hispanic* Americans

have experienced **discrimination** when seeking health care

Data source: 2021 Alzheimer's Disease Facts and Figures



On average, patients with rare diseases spend

$26,800

more than a patient with chronic disease.

Data source: National Economic Burden of Rare Disease

••• WEBINAR PANELISTS •••

Moderator:
Mr. Joff Masukawa

President, Diligentia Strategy

CHI Board of Directors

Ms. Sara Aswegan

Head of Rare Disease Commercial Asset Strategy , UCB

Dr. Mauvareen Beverley, MD

Patient Engagement and Cultural Competence Specialist

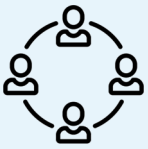
Ms. Jennifer Bright, MPA

Executive Director, Innovation and Value Initiative (IVI)

Mr. Dan Donovan

Founder & CEO, rareLife Solutions

BEST PRACTICES



Create Connectivity Among the Rare Disease Community

- Create interdisciplinary stakeholder dialogue to allow for discussions that need to take place to serve the rare community, regardless of where they're located.
- It is essential to listen to new perspectives to put learnings into action.
- Building a community and creating connectivity to have the conversations to enable change.

Understand the Concept of the Human Experience

- Create an environment of compassion, empathy, and inclusion for the patient, caregivers, and family.
- It's more important to know the patient who has the disease than the disease the patient has.
- If you don't know the individual, it will be difficult to help the individual with the disease.

Affirming language to reduce stigma

- Use the word disorder rather than the word disease
- Describe an individual who has a rare disorder as a person "living with xxx disorder" rather than " an xxx disease patient"
- Don't use the term "drug seeker." Labeling an individual living with a rare disorder as a drug seeker gives a negative connotation and could cause a provider to misunderstand what the individual needs.

Discover Ways to Act Together

- All organizations and individual sectors' perspectives are needed for solving these issues and making it standard practice for everyone.

Create a Transparent Learning Enterprise

- Data should be open access
- Allow patient perspectives from all organizations and individuals to be valid voices by opening up the pipeline of shared understanding and learning, so all sectors can accelerate and benefit.

Focus on 4 Key Areas of Trust, Accountability, Collaboration, and Sustainability

- Build trust
- Encourage and demand accountability
- Encourage collaboration and being a part of the collaboration
- Focus on long-term sustainability

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