



**NORD**<sup>®</sup>  
National Organization for Rare Disorders

# Compassionate Allowances and Rare Diseases

Marshall Summar, MD on behalf of NORD

Alone we are **rare**. Together we are strong.<sup>®</sup>



**NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.**



[rarediseases.org](http://rarediseases.org)

# More Than 30 Years of Service

- Advocacy – State & National
- Education – Patients, Caregivers & Medical Professionals
- Patient Assistance Programs
- Research Grants & Patient Registries







# Rare Disease Profile

- 7,000 diseases, each affecting fewer than 200,000 Americans
  - Many affect only a few hundred or a few thousand people
- 30 million Americans have rare diseases
- 95% of rare diseases have no treatment
- 80% have a genetic component
- Rare diseases tend to be serious, lifelong and life-altering
- Patients experience diagnosis delay, difficulty finding medical expert and lack of access to treatment or needed services

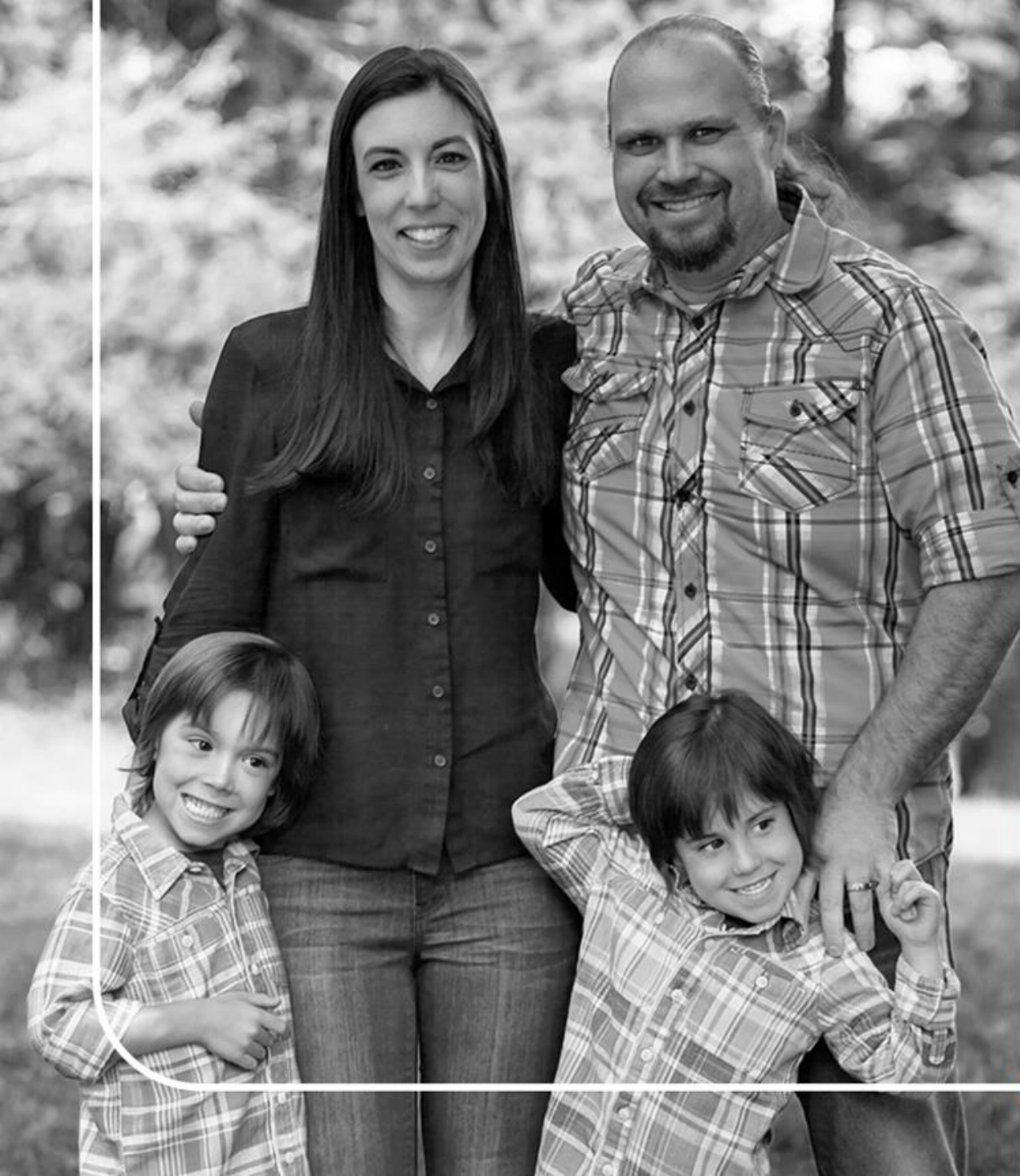
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# The Patient Experience

People with rare diseases often feel alone and abandoned. Many are not able to work or go to school. They experience social isolation and significant financial challenges. NORD serves as the voice of the rare disease community.



# International Collaboration

NORD works closely with its 260+ member organizations and advocacy partners to promote:

- Research
- Development of safe, effective treatments
- Patient access to care
- Fair and equitable public policies
- Education for patients, caregivers and medical professionals

NORD also collaborates with partners around the world, including:

- The European Organization for Rare Diseases (EURORDIS)
- Canadian Organization for Rare Disorders (CORD)
- Rare Diseases International
- Rare Voices Australia
- International Conference on Rare Diseases & Orphan Drugs
- Japan Patients Association
- International Rare Diseases Research Consortium
- Indian Organization for Rare Diseases



# Importance of Compassionate Allowances

NORD salutes SSA for its leadership in establishing the Compassionate Allowances Initiative several years ago.

This initiative is very important to patients and families affected by rare diseases.

Compassionate Allowances address one of the challenges of living with a rare, little-known medical condition.

NORD and our medical advisors are happy to support this initiative on behalf of the patients and families we serve.







Thank you.

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