

Infant Health Matters: Raising the Profile of Rare Diseases

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The National Coalition for Infant Health is a collaborative of more than 200 professional, clinical, community health, and family support organizations focused on improving the lives of premature infants through age two and their families. NCfIH's mission is to promote lifelong clinical, health, education, and supportive services needed by premature infants and their families. NCfIH prioritizes safety of this vulnerable population and access to approved therapies.

Thanks to a rapidly expanding movement, the rare disease community is finally getting a voice in state policy discussions.

Creating Rare Disease Advisory Councils

State legislatures create [Rare Disease Advisory Councils](#) to provide a unique opportunity for stakeholder engagement about important issues to the rare disease community. (1) In addition to elevating the policy conversation around rare diseases, some councils also conduct surveys and publish resources that are used to advance policy priorities.

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The word “rare” implies that these councils benefit only a few people. However, about 10% of Americans have a rare condition. This means 20-30 million patients’ interests are not being adequately represented in the policymaking process.

Councils’ objectives vary from state to state, but most are used to:

- Provide the rare disease community’s perspective in the policymaking process,
- Raise awareness about the 7,000 known rare diseases, and
- Identify barriers to appropriate care and propose solutions to improve access.

Patients, caregivers, and clinicians are commonly included and are valuable council members in that they provide a firsthand perspective about the challenges of living with a rare disease. Most councils have rare disease experts, researchers, and patient advocates. Some also include health plan representatives and policymakers.

About Rare Diseases

In the United States, a rare condition is defined as one that does not affect more than **200,000 people**. (2) Some of the most well-known are Huntington’s disease, spina bifida, fragile X syndrome, Crohn’s disease, cystic fibrosis, and Duchenne muscular dystrophy. Many other rare diseases, including post-transplant lymphoproliferative disease, IgG4-related disease, and amyloidosis, are not as well known, which can be increased by the work of Rare Disease Advisory Councils.

Most rare diseases are chronic and debilitating. Some are deadly. More than **80%** are caused by genetic variations that can strike at any age.

Many patients who have a rare condition spend years looking for clues and getting an accurate diagnosis. And even when they do, more than 90% of rare diseases have no FDA-approved treatment.

Patient advocates are optimistic that new energy brought on by the expanding network of Rare Disease Advisory Councils will lead to the discovery of breakthrough treatments and renewed support for the rare disease community. And creating a seat at the table for rare disease patients promotes the inclusion of their interests when health care policies are being considered.

References:

1. https://rarediseases.org/wp-content/uploads/2020/07/NRD-2049-State-Policy-Flyers-RDAC_v3-FINAL.pdf
2. https://www.cdc.gov/pcd/issues/2016/15_0491.htm

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National Coalition for Infant Health Values (SANE)

Safety. Premature infants are born vulnerable. Products, treatments and related public policies should prioritize these fragile infants’ safety.

Access. Budget-driven health care policies should not preclude premature infants’ access to preventative or necessary therapies.

Nutrition. Proper nutrition and full access to health care keep premature infants healthy after discharge from the NICU.

Equality. Prematurity and related vulnerabilities disproportionately impact minority and economically disadvantaged families. Restrictions on care and treatment should not worsen inherent disparities.