HCU NETWORK AMERICA

ADVOCACY TOOLKIT

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WHY ADVOCACY MATTERS FOR THE HCU COMMUNITY

Every federal and state policy decision related to medical nutrition, insurance coverage, newborn screening, or rare disease research directly affects individuals living with Homocystinuria (HCU). Without strong advocacy, policymakers may not fully understand the daily realities, medical risks, and lifelong management needs of HCU families.

HCU Advocacy Priorities

- Medical Foods and Medical Nutrition
- Newborn Screening
- Rare Disease Research

This toolkit equips HCU families, adults, caregivers, and clinicians to advocate for policies that strengthen access to medical foods, improve newborn screening systems, and support rare disease research.

KNOW THE LEGISLATORS AND THE ISSUE

Before contacting your legislators, preparation increases your impact.

What Legislators Respond To

- Personal stories from constituents directly affected by HCU.
- Clear, simple explanations of barriers (medical food coverage, access to specialized HCU care, insurance challenges).
- Data showing the financial impact of untreated or poorly managed disease.
- Consistent outreach from multiple constituents.

Find Your Legislators

- **Federal:** https://www.house.gov and https://www.senate.gov
- **State:** https://www.congress.gov/state-legislature-websites

Learn More About Newborn Screening & Medical Nutrition

- Baby's First Test (Newborn Screening): https://babysfirsttest.org/
- Patients & Providers for Medical Nutrition Equity (Medical Foods/Formula Advocacy): https://nutritionequity.org/

Review

- Committee assignments
- Positions on health care, rare diseases, and children's health
- Past support for medical nutrition legislation

Know Your Ask

Every communication should have:

- One clear request (e.g., "Please support legislation for medical nutrition coverage.")
- A short personal HCU story
- A clear explanation of how the issue affects HCU families

BUILDING RELATIONSHIPS OVER TIME

Effective advocacy is a relationship, not a single conversation.

Ways to Build Relationships

- Invite legislators to HCU webinars, community calls, or metabolic clinic visits.
- Share updates on HCU research, newborn screening, and patient stories.
- Attend town halls and district events.
- Become a trusted resource on metabolic disorders and medical nutrition needs.
- Thank legislators publicly when they support rare disease or medical food policy.
- Connect and tag them on social media when appropriate.

STAYING CONNECTED:
EMAIL, PHONE & SOCIAL MEDIA

Email

- Keep it personal, short, and direct. Clearly state your
- request in the first sentence. Include your address to
- verify you are a constituent.
- Attach your HCU Fact Sheet.

Phone Calls

- Especially effective during votes or committee activity.
- Share who you are, your connection to HCU, and your concrete ask.

Social Media

- Tag legislators when discussing HCU, medical foods, or rare disease issues.
- Raise awareness by sharing patient and caregiver stories.

INTRODUCE HCU NETWORK AMERICA AND YOUR STORY

Your HCU story is your strongest tool.

Share Your HCU Story

Briefly share:

- Where you live
- Your connection to HCU
- One key challenge (e.g., formula coverage, low-protein food access, newborn screening)
- Why policy change matters

Introduce HCU Network America

- A nonprofit supporting individuals and families living with HCU
- Provides education, family support, webinars, and resources
- Advances research and policy through collaboration with metabolic clinicians, researchers, and national rare disease coalitions

CONGRESSIONAL COMMITTEES MOST RELEVANT TO HCU

These committees have direct jurisdiction over medical nutrition coverage, newborn screening, HHS agencies, Medicaid/Medicare, and rare disease research.

Senate Finance Committee

https://www.finance.senate.gov

Senate Health, Education, Labor & Pensions (HELP) Committee

https://www.help.senate.gov

House Energy & Commerce Committee

https://energycommerce.house.gov

House Ways & Means Committee

https://waysandmeans.house.gov

Senate Appropriations Committee

https://www.appropriations.senate.gov

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https://www.appropriations.house.gov

TIPS FOR VIRTUAL OR IN-PERSON MEETINGS

Before the Meeting

- Send your HCU Fact Sheet with your meeting request.
- Coordinate your main speaking points and details of your personal story.
- Know the legislator's committee assignments and interests.
- Tip: Connect with the legislator's aide. Aides shape priorities and guide next steps, so building a relationship and serving as a reliable HCU resource can make a big difference.

During the Meeting

- Keep the tone respectful and focused.
- Share your HCU story early.
- Make a clear, specific ask.
- Offer to serve as a resource.

After the Meeting

- Send a thank-you note within 48 hours.
- Provide any follow-up materials requested.
- Maintain contact throughout the year.

ENGAGE LOCALLY: TOWN HALLS & COMMUNITY EVENTS

- Attend district town halls (virtual or in-person).
- Invite legislators to connect with HCU families or clinics.
- Organize small group discussions about medical nutrition needs.
- Ask your state legislators to support or establish a State Rare Disease Caucus (or rare disease working group).
- Advocate for state improvements to medical foods and formula coverage (benefit design, prior auth, reimbursement).
- Support state newborn screening program enhancements (funding, implementation, follow-up systems).

FIVE QUICK STEPS TO START TODAY

1. Look up your federal and state legislators

   Federal: *https://www.house.gov and https://www.senate.gov*
   State: *https://www.congress.gov/state-legislature-websites*

2. Sign up for their newsletters and follow them on social media

3. Email your story and your HCU Fact Sheet

4. Request a virtual or in-district meeting

5. Stay engaged regularly — advocacy builds over time

INTRACELLULAR COBALAMIN DISORDERS

A rare, inherited metabolic disorder requiring lifelong medical nutrition.

Quick Facts

Screening is dependent upon the state you are born in.

Requires high doses of hydroxocobalamin and betaine.

Illness, fasting, Nitrous oxide, or stress can trigger metabolic crises

Hydroxocobalamin is medically necessary, not optional

With proper management and access to care, individuals with intercellular cobalamin disorders can thrive

What is an Intracellular Cobalamin Disorder?

Intracellular cobalamin disorders are a group of rare, life-threatening genetic metabolic disorders that affect the body's ability to process vitamin B12. They are individually named Cobalamin C, D, E, F, G, J, K, and X. Individuals with these disorders cannot properly break down the amino acid homocysteine.

How can it affect the body?

The buildup of homocysteine levels can have dangerous and life-threatening impacts.

<u>High homocysteine levels may cause:</u>

- Severe vision impairment
- Global developmental delays
- Failure to thrive
- Seizures
- Anemia

How is it treated?

Intracellular cobalamin disorders are treated with a combination of hydroxycobalamin, betaine, and folinic acid. Treatment is required for life.

HCU
NETWORK
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About the HCU Network America

HCU Network America is a 501c3 nonprofit dedicated to:

- Supporting individuals and families living with HCU
- Providing education and resources
- Offering webinars, community connections, and family support
- Advocating for newborn screening and access to therapies
- Collaborating with metabolic clinics and rare disease partners

Why HCU Requires Policy Attention

HCU families face challenges that require legislative action:

- Medical Nutrition is an essential treatment, not optional
- Coverage for hydroxocobalamin varies by state and insurance plan
- Newborn screening saves lives—but states need strong screening systems and reliable follow-up
- Rare disease research accelerates better treatments and improves long-term outcomes

Key Challenges Facing HCU Families

- High out-of-pocket costs for hydroxocobalamin
- Inconsistent insurance coverage across states
- Variation in newborn screening, follow-up, and resources across states
- Limited rare disease research funding relative to need

How Public Policy Can Help

- Guarantee medical nutrition coverage under private insurance, Medicaid, and CHIP
- Include low-protein medical foods as part of covered treatment
- Strengthen and fund newborn screening programs
- Support rare disease initiatives and research through the National Institutes of Health (NIH) and the Health Resources and Services Administration (HRSA).
- In addition to federal action, advocates can often make faster progress at the state level on newborn screening, medical foods/formula coverage, and rare disease caucus efforts.

Learn More About Newborn Screening & Medical Nutrition

Newborn Screening: https://hcunetworkamerica.org/newborn-screening/

Patients & Providers for Medical Nutrition Equity (Medical Foods/Formula Advocacy): https://nutritionequity.org

HCU Network America
https://hcunetworkamerica.org

HCU Data Collection Program
https://rare-x.org/homocystinuria/