

## What happens with the right connections and advocacy?

Beyond being rare diseases, dystonia, pulmonary hypertension, and hereditary angioedema seem to have little in common. However:

- ✓ They all have robust federal engagement and partnerships with policymakers in key areas including medical research, treatment review and development (leading to multiple FDA approved therapies), and coverage and access.
- ✓ They have all worked with HMC for years to sustain efforts to advance research and improve care through grassroots outreach, scientific leadership, and advocacy.
- ✓ By connecting scientific breakthroughs and personal patient experiences with federal opportunities and various support mechanisms, they all have been able to make their voice heard and have been provided a seat at the table.



## The right advocacy strategy can help you:

- ✓ Further medical research for your rare disease community
- ✓ Help get treatments developed and approved
- ✓ Grow public awareness and professional education
- ✓ Improve health coverage and access to care or therapies



**Need professional guidance?**  
Scan the code or visit [hmcw.org](http://hmcw.org)



## A POCKET GUIDE TO RARE DISEASE ADVOCACY



**GET ORGANIZED.  
GET CONNECTED.  
GET RESULTS.**



## Most rare diseases still lack effective treatments. Changing this requires:

- ✓ Research and scientific advancements
- ✓ Treatment and development
- ✓ Ensuring coverage and access while lowering out-of-pocket costs for innovative treatment and care
- ✓ Collaboration with the appropriate agencies and elected officials

### The federal government provides the following resources at each stage of the process:

#### Research

- National Institutes of Health Rare Disease Clinical Research Network [rarediseasesnetwork.org/](http://rarediseasesnetwork.org/)
- NIH Categorical Spending by condition (including rare diseases): <https://report.nih.gov/funding/categorical-spending#/>
- DoD Congressionally Directed Medical Research Program <https://cdmrp.health.mil/>



#### Treatment Development

##### ClinicalTrials.gov

- FDA Patient Focused Drug Development Initiative [fda.gov/about-fda/office-clinical-policy-and-programs/patient-affairs-staff](http://fda.gov/about-fda/office-clinical-policy-and-programs/patient-affairs-staff)
- [www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development](http://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development)

##### Public Awareness and Professional Education

##### Centers for Disease Control and Prevention

- CDEA program [cdc.gov/populationhealth/chronicdiseaseawareness/funding-opportunities/expanding-education-approach-NOFO.html](http://cdc.gov/populationhealth/chronicdiseaseawareness/funding-opportunities/expanding-education-approach-NOFO.html)
- Health Resources and Services Administration
- Patient-Centered Outcomes Research Institute

#### Coverage, Access, and Out of Pocket Costs

- Medicare
- Medicaid
- Other Federal Programs
- Private Insurance Oversight

#### Regardless of politics, your elected officials need to be engaged:

- House.gov
- Senate.gov
- Appropriations Committee
- Energy & Commerce Committee/ HELP Committee
- Ways & Means Committee/ Finance Committee
- Constituent Services at Local Offices

With the right tools, small patient groups can have a big impact.

