

Rare Disease Legislation in the Queue

Rebuilding and Expanding the Rare Disease Congressional Caucus



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• What is a Caucus? •

Forum for **Members of Congress** to...

- **Voice** constituent concerns,
- **Educate** other Members and staff
- **Collaborate** on ideas
- **Facilitate** conversations between relevant stakeholders
- **Build** support for legislation

- **Mission Statement** •

“The Congressional Rare Disease Caucus is a forum for Members of Congress to voice constituent concerns, collaborate on ideas, facilitate conversations between the medical and patient community and build support for legislation that will improve the lives of people with rare diseases.”

• House Co-Chairs •



Rep. G.K. Butterfield (D-NC)



Rep. Leonard Lance (R-NJ)

• Senate Co-Chairs •



Sen. Orrin Hatch (R-UT)



Sen. Amy Klobuchar (D-MN)

• Just Ask •



Sen. Orrin Hatch (R-UT)

• Briefings •

Precision Medicine

Science Behind
Rare Disease Policy

Strengthening Medical Innovation in
America for Rare Disease Patients

The NIH and FDA:
Vital Agencies in the
Fight Against Rare
Diseases

Driving Innovation for
Lifesaving Therapies
through PDUFA
Reauthorization in 2017

21st Century Cures
Initiative: Priorities for
the Rare Disease
Community

The Rare Disease Ecosystem: Fostering Patient Engagement & Driving
Biomedical Innovation

Advancing Rare Disease Treatments in the Era of Cures and Health Care Reform

THURSDAY, MARCH 2ND AT HART SENATE OFFICE BUILDING ROOM 902

Lunch Briefing: 12:00pm – 1:30pm

Moderator: Caroline Kruse, Executive Director, **Platelet Disorder Support Association**

- Frank Sasinowski, Director, **Hyman, Phelps & McNamara PC** – “Implementing the 21st Century Cures Act”
- Eric Gascho, Vice President of Government Affairs, **National Health Council** – “The PDUFA Reauthorization Process in 2017”
- Beth McGinn, Director of Public Affairs and New Media, **ARTBA** – “The Affordable Care Act Repeal and Replacement”
- Clare Thibodeaux, Ph.D, MBA, Director of Scientific Affairs, **Cures Within Reach** – “New Models for Rare Disease Drug Development”
- “The Role of Incentives in the Development of Rare Disease Therapies”

• **Senate Membership** •

aka The Fab Five

Arkansas - Tom Cotton

Idaho - James Risch

Louisiana - David Vitter

Minnesota - Amy Klobuchar*

Utah - Orrin Hatch*

• House Membership •

100+

AL • CA • CO • CT • FL • GA
IL • IN • IA • KS • KY • IL • ME
MA • MI • MN • MS • NE • NV
NJ • NY • NC • NE • OH • OR PA
• RI • SC • TN • TX • VA
WA • WV

• House Membership •

MISSING

Alaska, Arizona, Arkansas, Hawaii, Idaho,
Louisiana, Maryland, Missouri, Montana,
New Hampshire, New Mexico, North
Dakota, Oklahoma, South Dakota, Utah,
Vermont, Wisconsin, Wyoming

• Ask •

Join the Rare Disease Caucus!