



Navigating Congress

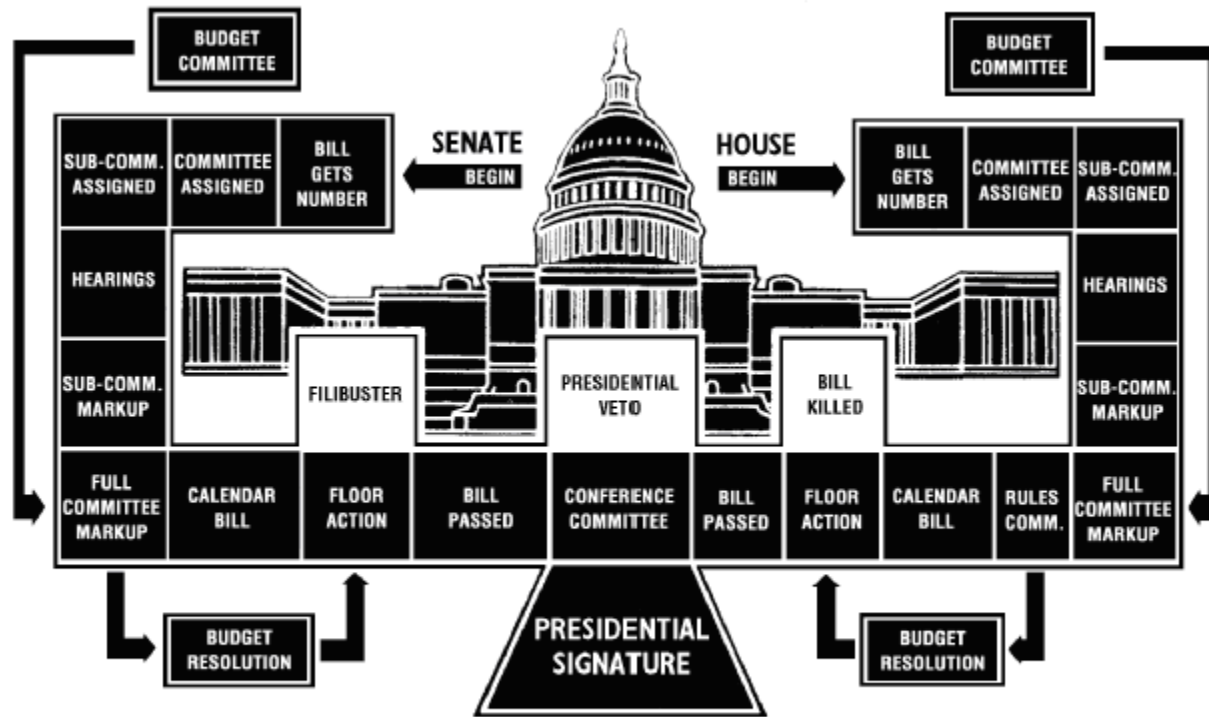
Includes Rare Disease
Legislative History

Making Sausage

- ▶ Otto von Bismarck: *“If you like laws and sausages, you should never watch either one being made.”*



Navigating Congress



113th Congress

- ▶ Groundhog Day. Again.



113th Congress

- ▶ 96 new Members
 - 84 in House of Representatives
 - 12 in the Senate
- ▶ Assume none of these new Members knows anything about rare diseases, FDASIA, or the Orphan Drug Act

113th Congress

Balance of Power The 113th Congress



Senate members



House members



Source: Bloomberg

113th Congress

- ▶ 101 Women Win!



113th Congress

*Medical Professionals, 113th Congress

House	Senate
Rep. Dan Benishek, M.D. (R- MI) – General Surgeon	Dr. John Barrasso, M.D. (R-WY) – Orthopedic Surgeon
Dr. Ami Bera, M.D. (D -CA) – Internist	Dr. Tom Coburn, M.D. (R-OK) – Family Practice
Dr. Charles Boustany, M.D. (R- LA) – Cardio Thoracic Surgeon	Dr. Rand Paul, M.D. (R-KY) – Ophthalmologist
Dr. Paul Broun, M.D. (R- GA) – Family Practice	
Dr. Larry Buschon, M.D. (R-IN) – Thoracic Surgeon	
Dr. Michael Burgess, M.D. (R-TX) – OB/GYN	
Dr. Bill Cassidy, M.D. (R-LA) – Gastroenterologist	
Dr. Donna Christensen, M.D. (D- VI) – Emergency Medicine	
Dr. Scott Desjarlais, M.D. (R -TN) – Family Practice	
Dr. John Fleming, M.D. (R- LA) – Family Practice	
Dr. Phil Gingrey, M.D. (R- GA) – OB/GYN	
Dr. Andy Harris, M.D. (R-MD) – Anesthesiologist	
Dr. Nan Hayworth, M.D. (R- NY) – Ophthalmologist	
Dr. Joe Heck, D.O. (R - NV) – Emergency Medicine	
Dr. Jim McDermott, M.D. (D- WA) – Psychiatrist	
Dr. Tom Price, M.D. (R-GA) – Orthopedic Surgeon	
Dr. Phil Roe, M.D. (R-TN) – OB/GYN	

Committees of Jurisdiction

- ▶ Most issues relevant to the rare disease community are under the jurisdiction of the House Energy & Commerce Committee and the Senate Health, Education, Labor & Pensions (HELP) Committee.
- ▶ There is a Health Subcommittee on both E&C and HELP

Energy & Commerce Committee Health Subcommittee Jurisdiction

Public health and quarantine; hospital construction; mental health; biomedical research and development; health information technology, privacy, and cybersecurity; **public health insurance** (Medicare, Medicaid) and **private health insurance**; medical malpractice and medical malpractice insurance; the **regulation of food, drugs, and cosmetics**; drug abuse; the **Department of Health and Human Services**; the **National Institutes of Health**; the **Centers for Disease Control**; Indian Health Service; and all aspects of the above-referenced jurisdiction related to the Department of Homeland Security.



Energy & Commerce Health Subcommittee Membership

Republican Members	Democratic Members
Joe Pitts (PA): Chairman	Frank Pallone, Jr (NJ): Ranking Member
Michael Burgess, MD (TX): Vice Chairman	John Dingell (MI)
Ralph Hall (TX)	Eliot Engel (NY)
Ed Whitfield (KY)	Lois Capps (CA)
John Shimkus (IL)	Jan Shakowsky (IL)
Mike Rogers (MI)	Jim Matheson (UT)
Tim Murphy (PA)	Gene Green (TX)
Marsha Blackburn (TN)	G.K. Butterfield (NC)
Phil Gingrey (GA)	John Barrow (GA)
Leonard Lance (NJ)	Donna Christensen (VI)
Bill Cassidy (LA)	Kathy Castor (FL)
Brett Guthrie (KY)	John Sarbanes (MD)
Morgan Griffith (VA)	Henry Waxman (CA)
Gus Bilirakis (FL)	
Renee Ellmers (NC)	
Joe Barton (TX)	
Fred Upton (MI)	

HELP Committee Membership

Democrats by Rank	Republicans by Rank
Tom Harkin (IA)	Lamar Alexander (TN)
Barbara A. Mikulski (MD)	Michael B. Enzi (WY)
Patty Murray (WA)	Richard Burr (NC)
Bernard Sanders (I) (VT)	Johnny Isakson (GA)
Robert P. Casey, Jr. (PA)	Rand Paul (KY)
Kay R. Hagan (NC)	Orrin G. Hatch (UT)
Al Franken (MN)	Pat Roberts (KS)
Michael F. Bennet (CO)	Lisa Murkowski (AK)
Sheldon Whitehouse (RI)	Mark Kirk (IL)
Tammy Baldwin (WI)	Tim Scott (SC)
Christopher S. Murphy (CT)	
Elizabeth Warren (MA)	

Senate HELP Committee

- ▶ The Committee has jurisdiction over several measures relating to education, labor, health and public welfare, including:
 - Aging
 - Biomedical research and development
 - Individuals with disabilities
 - Public health

Primary Health & Aging Subcommittee

Democrats by Rank	Republicans by Rank
Bernard Sanders (I) (VT)	Richard Burr (NC)
Barbara Mikulski (MD)	Pat Roberts (KS)
Kay Hagan (NC)	Lisa Murkowski (AK)
Sheldon Whitehouse (RI)	Michael Enzi (WY)
Tammy Baldwin (WI)	Mark Kirk (IL)
Elizabeth Warren (MA)	Lamar Alexander (TN) (ex officio)
Tom Harkin (IA) (ex officio)	

How to Lobby Congress

- ▶ **Keep your meeting brief**
 - 20 minutes
 - Who you are/your concerns
 - Try to make a connection between your issues and district impact
- ▶ **Have a well-defined “ask”**
- ▶ **Provide supporting information**
 - Leave a one-pager

How to Lobby Congress

- ▶ **Establish a relationship with the staff**
 - Be a reliable source of information
- ▶ **Send follow-up “thank you” email**
 - Send all correspondence via email, not US mail
- ▶ **If the Member/staff disagrees, find out why**
 - Is it the issue, or is it politics?

Potential Meeting Outcomes

- ▶ An office is supportive of your ask
- ▶ An office is non-committal
 - Do not press for a firm commitment
 - Ask if you can provide additional information
 - Follow-up via email
- ▶ An office disagrees with you
 - Find out why. Is it the issue or politics?
 - Is there merit to the opposition? Listen to their opinion
 - Can you moderate your ask?

The Orphan Drug Act

▶ What is an Orphan Drug?

- Orphan drugs are those that treat diseases affecting less than 200,000 Americans.

▶ Why Was the Orphan Drug Act Necessary?

- The market for orphan drugs is limited. To increase encourage the development of drugs for orphan diseases, the government created a number of incentives, including:
 - Seven year market exclusivity
 - Tax credits
 - Grants for drug development
 - Fast-track approvals of drugs for rare diseases
 - Expanded access to the investigational new drug program
 - Later amended to **waive user fees** charged as part of PDUFA
- The lead sponsor of the ODA was Rep. Henry Waxman, D-CA. It was signed into law by President Reagan in 1983. 2013 is the 30th anniversary of the ODA.



Two episodes of Quincy are credited with helping to pass the ODA.

The Orphan Drug Act

- ▶ Prior to enactment of the ODA in 1983:
 - Only 38 drugs were approved in the US to treat orphan diseases
- ▶ 1983–2010:
 - FDA approved 353 orphan drugs
 - Granted orphan designation to 2,116 compounds
- ▶ Of 7,000 rare diseases, fewer than 450 orphan products have been approved, treating only 250 rare diseases.

FDASIA Rare Disease Provisions

- ▶ **Accelerated Approval**
 - Fast Track designation and accelerated approval for “a broad range of serious or life–threatening diseases or conditions”
 - HIV/AIDS & Oncology most frequently accelerated approvals
- ▶ **Breakthrough Therapies**
 - Expedited development and review of drugs for serious and/or life–threatening diseases that show major improvement over the standard of care in phase 1/2 trials
- ▶ **Conflict of Interest**
 - No cap on conflict–of–interest waivers for FDA advisory committee members

FDASIA Rare Disease Provisions

▶ Grants & Contracts

- Reauthorizes the orphan product grants program for another five years from 2013 through 2017

▶ Priority Vouchers

- Priority Review voucher program to rare pediatric diseases

▶ Rare Disease Experts

- FDA to establish list of rare disease experts whom FDA may consult



Useful Resources

- ▶ **Federal Register**
 - www.federalregister.gov
 - Notices/rulemaking
- ▶ **US House of Representatives Website**
 - www.house.gov
 - Link to Member offices, committees & subcommittees
- ▶ **US Senate Website**
 - www.senate.gov
 - Link to Member offices, committees & subcommittees
- ▶ **HHS Website**
 - www.hhs.gov
- ▶ **FDA Website**
 - www.fda.gov
- ▶ **Congressional Research Service Reports**
 - www.opencrs.com