



# Under the Dome – Rare Disease Advocacy

Rare Bootcamp | PGPA Overview

**November 2024**

# The Team



**Betsy  
Ricketts**

**Vice President**  
*Policy, Government  
and Public Affairs*  
**Novato HQ**



**Lisa  
Kahlman**

**Executive Director**  
*Public Policy and  
Public Affairs*  
**Portland, OR  
(Field)**



**Julie  
Boyd**

**Senior Director**  
*State Government Affairs  
(West)*  
**Austin, TX  
(Field)**



**Erin  
Frey**

**Senior Director**  
*State Government Affairs  
(East)*  
**Raleigh-Durham, NC  
(Field)**



**Isabel  
Bueso**

**Public Affairs Specialist**  
*State Government Affairs  
(CA)*  
**Novato HQ**

# Policy, Government, & Public Affairs

## 2025 High-Level Priorities

---

1

Develop conducive global political environment to **support ability of UGX to do business and support access & reimbursement** of our key products

2

Lead and support **rare disease initiatives** on the federal and state levels

3

Advance **earlier diagnosis initiatives** at the federal and state levels

4

Build global reputation as **sought after thought partner and good neighbor** with policy, patient, community and political leaders

# 2025 Global Policy Priorities (to be approved by GPC and XLT)

---

- ❑ **Patient Experience** – Lead efforts to put patients at the forefront from development through commercialization (e.g., have patient experience data included in drug applications and labels)
- ❑ **Market Access** – Ensure that all rare diseases are treated as a priority for world-wide markets.
- ❑ **Pricing & Reimbursement** – Ensure majority access to our products through proactive payer engagement, education, and advocacy on clinical unmet need and responsible pricing philosophy; ensure flexibility in payment models for innovative therapies, underpinning sustainability of healthcare systems and innovation
- ❑ **Diagnostic Odyssey** – Lead efforts to shorten the timeline from diagnosis to access of approved therapies (e.g., newborn screening, genomic sequencing, diagnostic codes, etc.)
- ❑ **Gene Therapy** – Engage with policy makers in innovative new thinking and approaches in overall clinical and manufacturing developments leading to approval and set the stage for launches by characterizing unmet need and burden of illness in peer-reviewed literature.
- ❑ **Accelerated Development** - Streamline rare disease drug development by engaging with Health Authorities globally to shorten non-clinical / clinical / CMC development timelines thereby ensuring speedier assessment and approval including efforts around biomarkers, endpoint development, etc.
- ❑ **Emerging Markets** – Raise awareness of ultra-rare diseases and encourage local definitions around “rare”, “ultra-rare” and “orphan” as well as educate re advanced therapies (e.g., gene therapy, mRNA)

# Political Environment in Which Government Affairs is Operating in 2024

---

## U.S. Federal Government

### Decision Making Bodies:

*White House, Health and Human Services, FDA, Senate Finance Committee, Senate Health Education Labor and Pension Committee, House Energy and Commerce, Key State Legislators and District Reps, Rare Disease Caucus members*

### Hot Topics:

New Administration Transition, Budget Constraints, Increase Health Coverage, Drug Pricing

## State Governments

### Decision Making Bodies:

*Governor's Office, Health Department Secretary, Medicaid Director, Senate and House Leadership, Senate and House Budget and Health Committee Chairs and Members, Key District Legislators, Rare Disease Councils, Biotech Caucus, Joint Budget Committees*

### Hot Topics:

Budget Constraints, High-Cost Drug Coverage

# Ultragenyx Key Issues and Key States

---

## Federal

- Healthcare Budgets and Access
- FDA Reforms
- Priority Review Voucher Reauth
- IRA Legislation/Drug Prices
- Rare Disease Caucus Issues
- Newborn Screening Reauthorization
- Ultrarare Incentives

## State

- Budget – line item, Medicaid reimbursements
- Pricing/transparency
- Newborn Screening
- Rare Disease Caucus
- Rare Disease Advisory Councils
- Value Based Arrangements – Pay for Gene Therapy

# Ultragenyx Elected Official Representatives

Governi ng Entity	Novato Office	Brisbane Office	Miami Office	Utah Office	Woburn Office	Bedford Facility	Sommerville Office	Texas Office
<b>U.S. Senate</b>	Sen.-Elect Adam Schiff (D)	Sen.-Elect Adam Schiff (D)	Sen. Rick Scott (R)	Sen.-Elect John Curtis (R)	Sen. Ed Markey (D)	Sen. Ed Markey (D)	Sen. Ed Markey (D)	Sen. Ted Cruz (R)
	Sen. Alex Padilla (D)	Sen. Alex Padilla (D)	Sen. Marco Rubio (R)	Sen. Mike Lee (R)	Sen. Elizabeth Warren (D)	Sen. Elizabeth Warren (D)	Sen. Elizabeth Warren (D)	Sen. John Cornyn (R)
<b>U.S. House</b>	Rep. Jared Huffman (D- 2)	Rep. Kevin Mullin (D-15)	Rep. Maria Elvira Salazar (R-27)	Rep. Celeste Maloy (R-2)	Rep. Katherine Clark (D-5)	Rep. Seth Moulton (D- 6)	Rep. Ayanna Pressley (D-7)	Rep. Michael McCaul (R-10)

# Ultragenyx Elected State Representatives

Governing Entity	Novato Office	Brisbane Office	South San Francisco Office	Somerville Office	Woburn Office	Bedford Facility	Miami Office	Texas Office	Utah Office
<b>Governor</b>	Gov. Gavin Newsom (D)	Gov. Gavin Newsom (D)	Gov. Gavin Newsom (D)	Gov. Maura Healey (D)	Gov. Maura Healey (D)	Gov. Maura Healey (D)	Gov. Ron DeSantis (R)	Gov. Greg Abbott (R)	Gov. Spencer Cox (R)
<b>State Senate</b>	Sen. Mike McGuire (D-2)	Sen. Josh Becker (D-13)	Sen. Josh Becker (D-13)	Sen. Patricia Jehlen (D – 2 <sup>nd</sup> Middlesex)	Sen. Cindy Friedman (D-4 <sup>th</sup> Middlesex)	Sen. Michael Barrett (D-3 <sup>rd</sup> Middlesex)	Sen. Ileana Garcia (D – 36 <sup>th</sup> )	Sen. Charles Schwertner (R-5)	Sen. Derek Kitchen (D-2)
<b>State Assembly</b>	Assm. Damon Connolly (D-10)	Assm. Diane Pappan (D-22)	Assm. Diane Pappan (D-22)	Rep. Mike Connolly (D-26 <sup>th</sup> Middlesex)	Rep. Richard Haggerty (D-30 <sup>th</sup> Middlesex)	Rep. Ken Gordon (D-21 <sup>st</sup> Middlesex)	Rep. Demi Busatta Cabrera (D – 114 <sup>th</sup> )	Rep. John Raney (R-14)	Rep. Dailey-Provost (D-24)
<b>County Supervisor</b>	Sup. Eric Lucan (Dist. 5)	Sup. David Canepa (Dist. 5) (San Mateo)	Sup. David Canepa (Dist. 5) (San Mateo)	(Middlesex) – no county government	(Middlesex) – no county government	(Middlesex) – no county government	Rebeca Sosa (Dist. 6)	Commissioner Nancy Berry (Precinct 3)	Arlyn Bradshaw (Dist. 1)
<b>City Mayor</b>	Mayor Mark Milberg (2024)	Mayor Coleen Mackin	Mayor Mark Nagales	Mayor Katjana Ballantyne (D)	Mayor Scott Galvin	Village Manager Matt Hanson	County Mayor Daniella Cava (D)  City Mayor Francis Suarez (R)	Mayor Karl Mooney	Mayor Erin Mendenhall




# Federal Coalition Engagements


---



Alliance for  
Regenerative Medicine



BIO



EveryLife Foundation  
community congresses



Institute for  
Gene Therapies



NORD  
Corporate Council



Rare Disease Company  
Coalition

# Leadership on Industry Associations

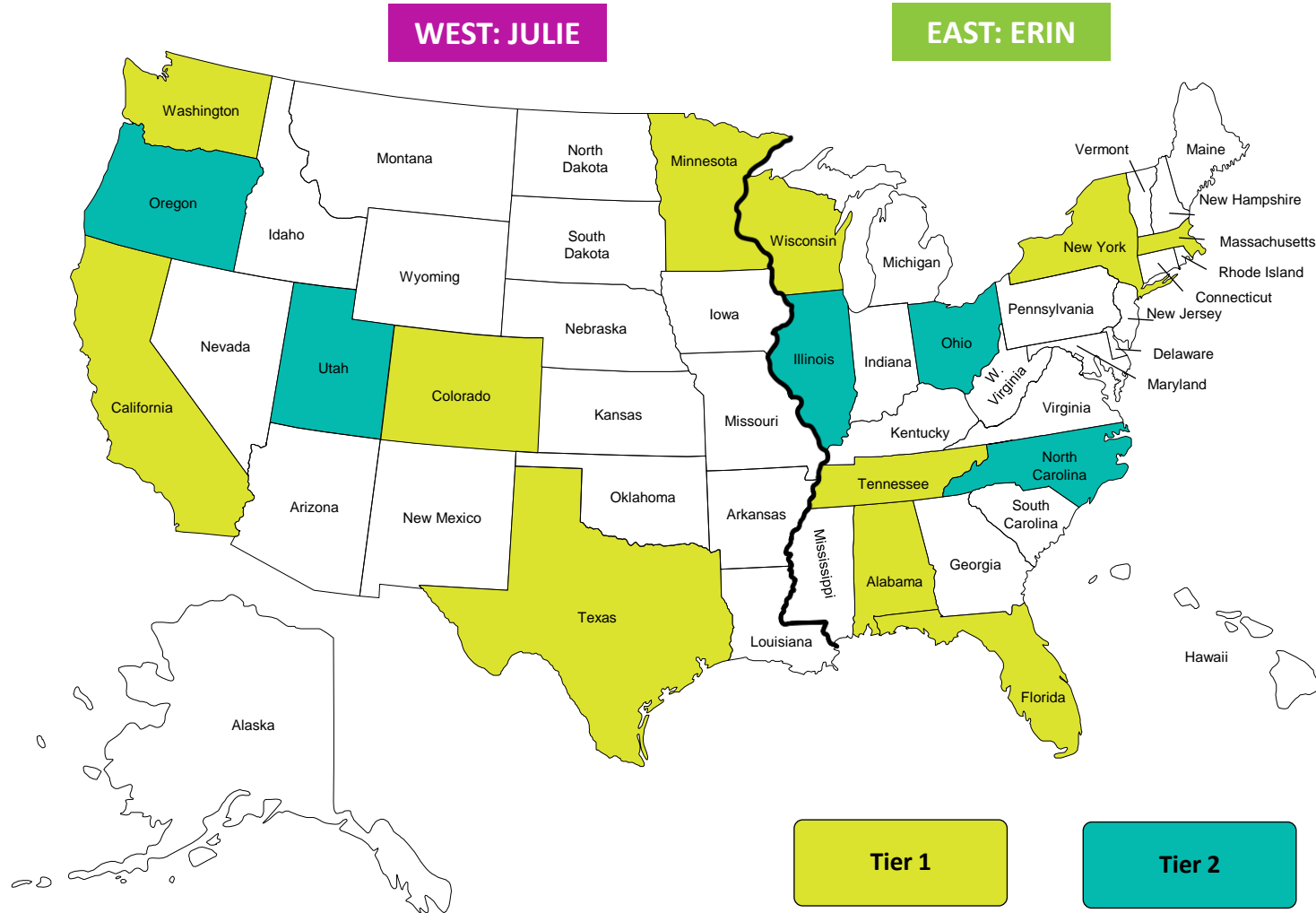
Organization	Representative(s)
Alliance for Regenerative Medicine Board Member	Karah Parschauer
California Life Sciences Board Member	Erik Harris
MassBio Board Member	Dennis Huang
Rare Disease Company Coalition (Board member and Co-Chair of Federal Policy Committee)	Lisa Kahlman

# State Governing Landscape

## Relevant Decision-Making Bodies

- Governors' offices
- Health Department Secretary/Commissioners
- Drug Utilization Review (DUR) Boards/Pharmacy & Therapeutics (P&T) Committees
- Medicaid Directors
- Senate and House leadership
- Senate and House Budget and Health Committee Chairs and members
- Key state legislators and district representatives
- Rare Disease Advisory Councils
- Rare Disease, Biotech and Life Science Caucus members

# Government Affairs Key States for 2025

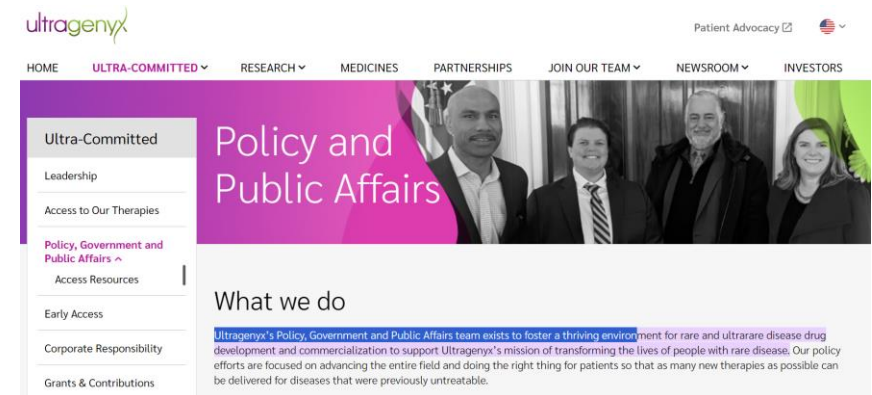


# Rare Disease Advisory Councils by State



# Ultragenyx Resources

- [PGPA Policy Page](#)
- [Newborn Screening Resources](#)
- [Patient Advocacy Website](#)



## Opportunities to Engage – PAG Advocacy Organizations

---

- EveryLife Foundation – Community Congress, Rare Disease Day, Rare Across America
- NORD – State Councils, Proclamations
- Global Genes – Community Conferences
- Institute for Gene Therapy – Patient Advisory Council
- State Rare Disease Advisory Councils
- Zebra Network

# Questions

---



# Additional Resource

---



[Find and Contact Elected Officials](#)