



**CELL**  **GENE**  
CONGRESSIONAL FLY-IN

## **Advocate Training Webinar**

June 27, 2024





## Webinar Agenda

- **ARM Mission & Public Affairs Strategy**
- **Fly-In Overview**
- **ARM's Policy Platforms**
- **Fly-In Legislative Asks**
- **What to Expect on Lobby Day**
- **Advocacy Best Practices**

# ARM is the Global Voice of the Cell & Gene Therapy Sector

The Alliance for Regenerative Medicine is the **leading international advocacy organization** championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society.

**Patients are our north star.**

## OUR FOCUS



Convening the sector



Advancing the narrative with data and analysis



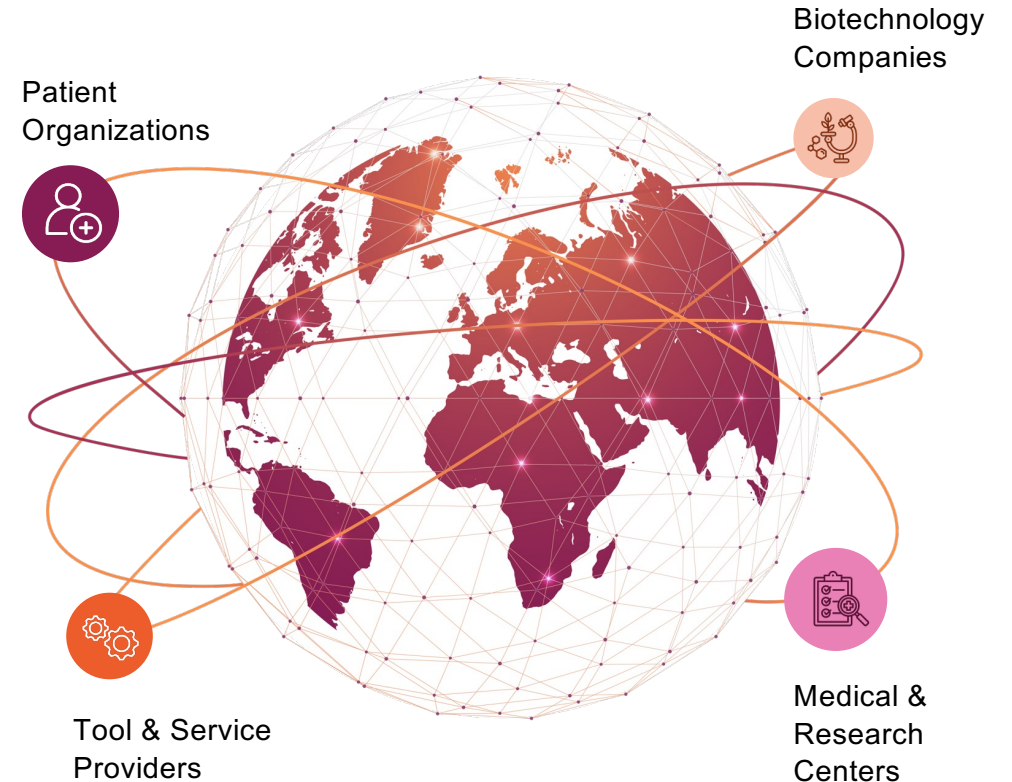
Engaging key stakeholders



Enabling the development of advanced therapies



Modernizing healthcare systems



**Representing 400+ members worldwide**

# ARM's Public Affairs Strategy

## THE "WHAT"



1. Achieve patient access in the payer and regulatory domain



2. Achieve adequate reimbursement for innovative technology

## THE "HOW"

01

### Building the Future Of Medicine

Convening the sector and facilitating influential exchanges on policies and practices

02

### Modernizing US reimbursement:

ARM is shaping the innovation ecosystem within Medicaid and Medicare

03

### FDA redesign:

Once-in-a-generation moment to support FDA capabilities

04

### Challenging European access:

'Moment of truth' with value recognition

05

### Voice of the Sector:

Building the Alliance's reputation through unique narratives, data /analysis outputs and executive engagement

06

### Leadership:

ARM has built a robust governance system to engage members and deliver its ambitious agenda



# Fly-In Overview

# Why Does ARM hold an annual Congressional Fly-In?



... so our members can

## Learn

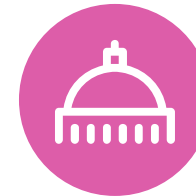


Dive deeper into ARM's policy objectives

Hear legislative and regulatory updates directly from policymakers

Understand federal policymaking process

## Influence

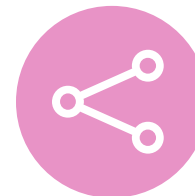


Educate lawmakers on CGTs and share invaluable on-the-ground perspectives

Advance ARM's legislative priorities through direct advocacy

Forge relationships with lawmakers

## Network



Meet & reconnect with other ARM members

Engage with ARM's Board of Directors, ARM Advisory Group Leaders, and ARM staff



# Fly-In Impact on Congress

Last year, ARM Fly-In attendees met with 83 Congressional offices and

- Increased interest in and awareness of CGTs (as reported by Congressional staff)
- Added additional co-sponsors to the MVP Act
- Solidified support for the MVP Act among House Energy & Commerce Committee members, ensuring it was voted out of Committee weeks later
- Added new members to the Congressional Personalized Medicine Caucus



## Latest News

### Guthrie's MVP Act Passes Full Committee

Washington, May 24, 2023 | [S.K. Bowen](#) (202-225-3501)  
Tags: [Health Care](#)

WASHINGTON, D.C. - House Energy and Commerce Committee Subcommittee on Health Chair Brett Guthrie (KY-02) released the following statement praising the passage of the *Medicaid VBPs for Patients (MVP) Act* through the full Energy and Commerce Committee.

"The Medicaid VBPs for Patients Act would help get life-saving treatments to the most vulnerable patients across the country. With the flexibility this legislation provides, states can make high-cost therapies and cures for rare diseases available without raising taxes or cutting other state programs. Value-based payments ensure states are not on the hook for paying a drug manufacturer for a high-cost treatment if it is not effective and can even save states money in the long-term in caring for a patient.



ENDPOINTS NEWS CHANNELS WEBINARS BIOPHARMA JOBS MORE

February 12, 2024 03:58 PM EST | Pharma, FDA+

## Stakeholders brace for bumps in rare pediatric disease PRV reauthorization

Lia DeGroot

A voucher program designed to stimulate drug development for rare pediatric diseases will sunset later this year if lawmakers don't reauthorize it — and rare disease advocates and experts are bracing for bumps in the road to renewal.

This year, attendees will have *even more* Congressional meetings and have the opportunity to

- Continue building support for the now bicameral MVP Act
- Add co-sponsors to the Accelerating Kids' Access to Care Act which was recently voted out of the Energy & Commerce Committee
- Educate Congressional staff on the importance of the Pediatric Rare Disease Priority Review Voucher at a critical time before the program's authorization expires in September



# Event Schedule

## Monday, July 15

Time	Session
9:30am - 12:00pm	U.S. Policy & Advocacy Forum Meeting
12:30pm	Event check-in opens
1:30 - 2:00pm	<b>Keynote:</b> Stephanie Heathman, M.S., Opie Jones Foundation
2:00 - 2:30 pm	<b>Keynote:</b> Dr. Nicole Verdun, Director, Office of Therapeutic Products, FDA
2:30 - 3:00 pm	Stakeholder Panel: Congressional staff
3:00 - 4:10 pm	2024 Election Outlook Panel
4:15 - 4:45 pm	Capitol Hill Day Brief (Overview of legislative “asks”)
4:45 - 5:15 pm	Congressional Team Huddles
6:00 - 9:00 pm	Reception & Dinner

## Tuesday, July 16

Time	Session
7:30 - 8:30 am	Breakfast & Legislative Briefing
9:00 - 4:00 pm	Lobby Day (meetings on Capitol Hill)
5:00 - 7:00 pm	Lobby Day Reception



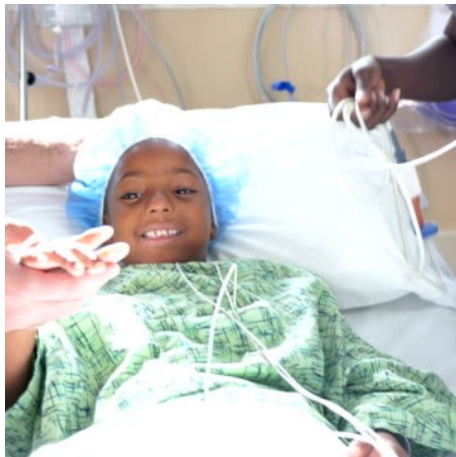




# **ARM's Policy Platforms**

## Address Medicaid Access Barriers

Eliminate coverage delays, ensure adequate reimbursement, streamline provider credentialing, enable patient support programs



## Support VBA Adoption

Facilitate value-based contracting by addressing regulatory and legal barriers



## Improve Regulatory Environment

Supporting FDA's office of Therapeutic Products (OTP) and ensuring the use and effectiveness of FDA programs and pathways





# **2024 Fly-In Legislative Asks**

# Accelerating Kids' Access to Care Act

S. 2372/H.R. 4758

## Background

Given the highly specialized nature of CGTs, manufactures often have limited treatment centers on board to provide care. It is not uncommon for patients to travel to an out-of-state treatment center for CGTs, creating an administrative challenge in provider credentialing and potentially delaying necessary care.

## The Accelerating Kids' Access to Care Act would

- Introduce a streamlined process for doctors to enroll in another state's Medicaid program by creating a new pathway for pediatric providers to enroll in multiple state Medicaid programs
  - Must meet specific requirements such as (1) being in the lowest category for potential program integrity issues or (2) enrolled in their home state's Medicaid program
- Focus only on provider screening and enrollment and not on the authorization of care by an out-of-state provider or payment rates for such care

## Impact

**Improve children's timely access to care** and **eliminates administrative burdens** and delays for providers and states

**Ask: Cosponsor the bill** (if they are not already co-sponsors)



# Accelerating Kids' Access to Care Act

S. 2372/H.R. 4758

## Legislative Activity

- 116 co-sponsors in the House and 37 in the Senate
- On June 12, the House Energy and Commerce Committee unanimously passed the bill, marking a key step toward its enactment.
- Recent positive discussions with the Congressional Budget Office (CBO) resulted in a new score of \$218 billion over ten years.

## ARM's Work

- Issued multiple letter of support
- Met with key leaders in the House to express support
- Met with CMS to discuss concerns
- Media engagement
- Congressional Briefings

## Stakeholder Support

Over 200 organizations supporting

- Children's Hospital Association
- American Academy of Pediatrics
- Patient Groups, including EveryLife Foundation, Leukemia & Lymphoma Society, National Organization for Rare Disorders (NORD)
- ASTCT



September 25, 2023

The Honorable Charles Grassley  
United States Senate  
135 Hart Senate Office Building  
Washington, DC 20510

The Honorable Michael Bennet  
United States Senate  
261 Russell Senate Building  
Washington, DC 20510

The Honorable Lori Trahan  
United States House of Representatives  
2439 Rayburn House Office Building  
Washington, DC 20515

The Honorable Mariannette Miller Meeks  
United States House of Representatives  
1034 Longworth House Office Building  
Washington, DC 20515

Re: Accelerating Kids' Access to Care Act

Dear Senators Grassley and Bennet and Representatives Trahan and Miller Meeks:

On behalf of the Alliance for Regenerative Medicine (ARM), I thank you for your leadership in championing policies to ensure children with medically complex conditions enrolled in Medicaid have access to specialized care across state lines.

ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. We represent more than 400 emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

In recent years, numerous transformative – and sometimes life-saving – cell and gene therapies

(CGTs) have been approved for difficult-to-treat conditions such as adrenoleukodystrophy, muscular dystrophy, and sickle cell disease. In the US to test the next generation of children or individuals with these conditions, two gene therapies for sickle cell disease must be approved by the FDA

EXCLUSIVE

## Texas Medicaid agrees to fully cover gene therapy for Afghan refugees' infant



By Megan Molteni Feb. 5, 2024

Reprints

ARM is committed to ensuring access to these therapies. However, in some highly specialized geographic areas. We support



# Medicaid Value-Based Payments for Patients (MVP) Act

H.R. 2666/S.4204

## Background

VBP can defray the upfront cost of CGTs, supporting state Medicaid agencies as they manage their budgets. However, the current system was not designed for these types of arrangements, nor one-time administered, durable therapies.

## The MVP Act would

- Codify Medicaid “Best Price (BP) Rule” that allows developers to report different BPs for VBPs and traditional pricing.
- Clarify how two commonly used pricing metrics (average sales price and average manufacturer price) are reported for VBP arrangements
- Allow for VBP payment without running afoul of anti-kickback law
- Require a study on the effectiveness of VBPs on patient access and health system costs

## Impact

The MVP Act will **encourage the use of VBPs for CGTs**, expanding access to curative treatments. Many states are interested in pursuing VBP models to **provide access to patients with Medicaid** (which insures a disproportionate number of rare diseases patients)

**Ask: Cosponsor the bill** (if they are not already co-sponsors)



# MVP Act

# H.R. 2666/S.4204

## Legislative History

- Bill first introduced in the 117<sup>th</sup> Congress
- Driven by advocacy and approval of high-profile therapies, the bill was reintroduced in the 118<sup>th</sup> Congress
- House bill was advanced through the full House E&C Committee in 2023
  - Current Cosponsors: 20 Democrat, 19 Republican
- Senate bill introduced in April 2024
  - Current Cosponsors: 2 Democrat, 2 Republican

## ARM's Work

- Encouraged bill sponsors (House and Senate) to draft and introduce legislation
- Provided technical feedback on bill text prior to introduction & during mark-up
- Issued letter of support & submitted hearing testimony
- Met with myriad Members of Congress to encourage co-sponsorship
- Media engagement (e.g., interviews, news stories, op-ed)
- Congressional Briefings

## Stakeholder Support

- Patient Groups (including EveryLife Foundation, Sickle Cell Disease Partnership, 20+ rare disease advocacy organizations)
- ASGCT
- Institute for Gene Therapy (IGT)
- Academy of Managed Care Pharmacy
- Council for Affordable Health Coverage

## ARM Releases Statement on MVP Act Re-Introduction

Washington, DC – April 20, 2023

The Alliance for Regenerative Medicine (ARM) applauds the bipartisan effort by Representatives Guthrie (R-KY), Eshoo (D-CA), Joyce (R-PA), Auchincloss (D-MA), Miller-Meeks (R-IA), and Peters (D-CA) for introducing H.R. 2666, the "Medicaid VBPs for Patients (MVP)" Act. Advancing value-based payment arrangements for cell and gene therapy has been a long-standing priority for ARM, and we view this as a step in the right direction to promoting access to cell and gene therapies for the Medicaid patients that need them.

We look forward to continuing our work with the bill sponsors, and their colleagues in the Senate, to improve the value-based payment landscape through meaningful legislative reform that supports patient access to transformative cell and gene therapies.



August 15, 2023

The Honorable Brett Guthrie  
U.S. House of Representatives  
Washington, DC 20515

The Honorable Anna Eshoo  
U.S. House of Representatives  
Washington, DC 20515

Dear Representatives Guthrie and Eshoo,

On behalf of the Alliance for Regenerative Medicine and established biotechnology companies, academic organizations, I thank you for your leadership championing durable, potentially curative cell and gene therapies

CGTs are at the forefront of the fight against some of the most devastating diseases. CGTs will soon be available for both rare and common disorders. To this end, it is particularly important to ensure that these therapies are accessible to all who need them, as Medicaid will likely be a prominent payer for these therapies in the coming years.

We strongly support your *Medicaid VBPs for Patients* Act introduced by Representatives Auchincloss (D-MA), Miller-Meeks (R-IA), Crenshaw (R-TX), Billirakis (R-FL), Dunn (R-FL), Eshoo (D-CA), and Thompson (D-CA).

Value-based payment (VBP) arrangements can help Medicaid agencies as they manage their budgets. Supporting these arrangements ensures that states are only paying for products that provide the most value to patients.

The MVP Act helps facilitate these types of VBPs by providing a framework for a Medicaid program on its effectiveness rather than the quantity of medicine consumed. The MVP Act would encourage the use of VBAs by states and manufacturers by codifying recent CMS changes to the Medicaid Best Price (BP) regulations. The legislation also makes critical changes to other legacy provisions that were codified well before CGTs were a reality in order to accommodate VBPs, including those related to the calculation of Average Sales Price (ASP), Average Manufacturer Price (AMP), as well as provisions of the Federal Anti-Kickback Statute (AKS).

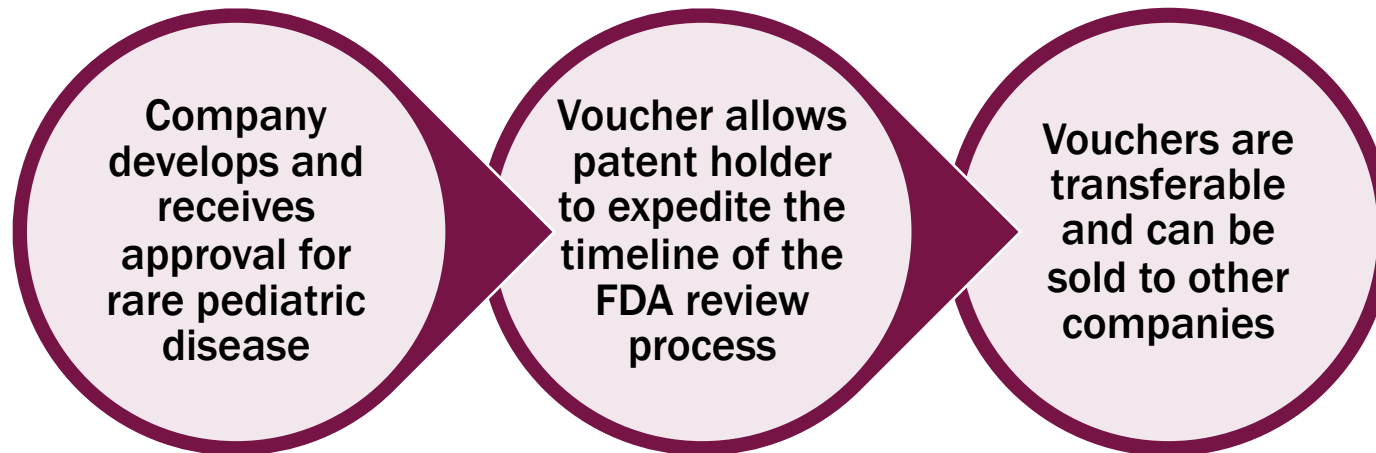
Many changes were made to the introduced bill during the Committee process to respond to concerns about scope, product prices, and costs to states. In addition, the bill requires a GAO study on the effectiveness of VBPs on patient access and overall health system costs related to "transformative



# Pediatric Rare Disease Priority Review Voucher (PRV) Reauthorization

## Background

Children comprise as many as half of those living with rare diseases, yet their treatment options are extremely limited. The Creating Hope Reauthorization Act looks to address this issue by reauthorizing and expanding the cost-neutral FDA priority review voucher (PRV) program, which incentivizes manufacturers to develop products for rare pediatric indications by expediting FDA review. **The program is set to expire on September 30<sup>th</sup>, unless reauthorized by Congress.**



## Impact

- This program has led to innovation addressing unmet medical needs across 47 rare pediatric indications and benefitted more than 200,000 rare disease patients
- More than half of all PRVs were granted in just the last four years, and more than 90% of all PRVs have been awarded to treatments for indications with no approved therapy on the market

**Ask:** Co-sponsor the Give Kids a Chance Act (H.R. 3433/S. 2897)





# Give Kids a Chance Act

H.R. 3433/S. 2897

## Legislative History

- The program was initially established by the Creating Hope Act of 2012 and has been reauthorized multiple times
- Unless reauthorized, **set to expire September 30**
- House Energy and Commerce Committee will consider a 5 or 6 year reauthorization in the coming weeks.
- Current Cosponsors
  - House bill: 16 Democrat, 22 Republican
  - Senate bill: 5 Democrat, 1 Republican

## ARM's Work

- Issued letter of support & submitted hearing testimony
- Met with key leaders in the House to express support

## Stakeholder Support

- Multiple Children's Hospitals
- EveryLife Foundation
- National Organization for Rare Disorders
- Rare Disease Company Coalition
- BIO





# **What to Expect on Lobby Day**

# Advocacy Materials in Your Folder

## Personal Hill meeting schedule

- Includes key information about each member of Congress and office location

### Confirmed Meetings

7:30am	Breakfast Reserve Officers Association on Capitol Hill 1 Constitution Ave NE Top Floor - Top of the Hill Venue
9:30 a.m.	Meeting with Quentin Dupouy, Legislative Assistant Office of Rep. Jared Huffman (D-2-CA) 2445 Rayburn House Office Building; 202.225.5161 <a href="#">Ultragenyx is located in Novato, CA</a>
10:30 a.m.	Meeting with Maddy Hanley, Legislative Assistant Office of Rep. Judy Chu (D-28-CA) <i>House Ways &amp; Means Committee</i> 2423 Rayburn House Office Building; 202.224.5464 <a href="#">Ahsan is Constituent</a>
11:00 a.m.	Meeting with Alison Feinswog, Senior Legislative Assistant Office of Rep. Mike Levin (D-49-CA) <i>Personalized Medicine Caucus</i> 2352 Rayburn House Office Building; 202.225.3906

## Postcard with QR code

- Leave behind for Hill staffer – QR code links to bill one-pagers and ARM website



## One-page ARM overview

- Attendees will have a hard copy for reference
- Includes information about ARM members and recent cell and gene therapy sector data

## One-page background on each bill (3 total)

- Attendees will have a hard copy of each one-pager to use for reference
- ARM will share background materials electronically with Hill staffer after your meeting

## Talking points for each bill (3 total)

- Suggestions to aid your discussion of each bill
- For your use only; DO NOT share document with Hill staffer

## FAQ document

- Includes general information & answers to common questions from Hill staffers
- For your use only; DO NOT share document with Hill staffer



## Congressional Teams



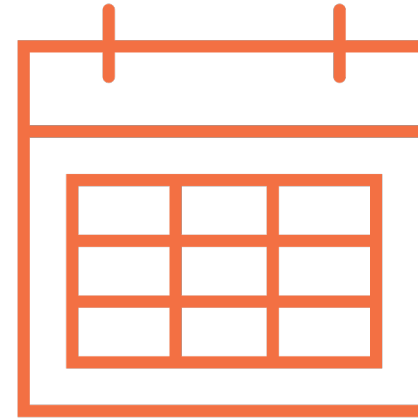
Teams formed based on

- Your home constituency and/or
- Location of your company/facilities
- Any pre-existing relationships with a member of Congress or expertise of special interest to a member of Congress

Congressional staff want to hear about issues from constituents

Every team has a **team lead** who is an ARM staff member or ARM consultant

## Meeting Schedule



Hill meetings scheduled to

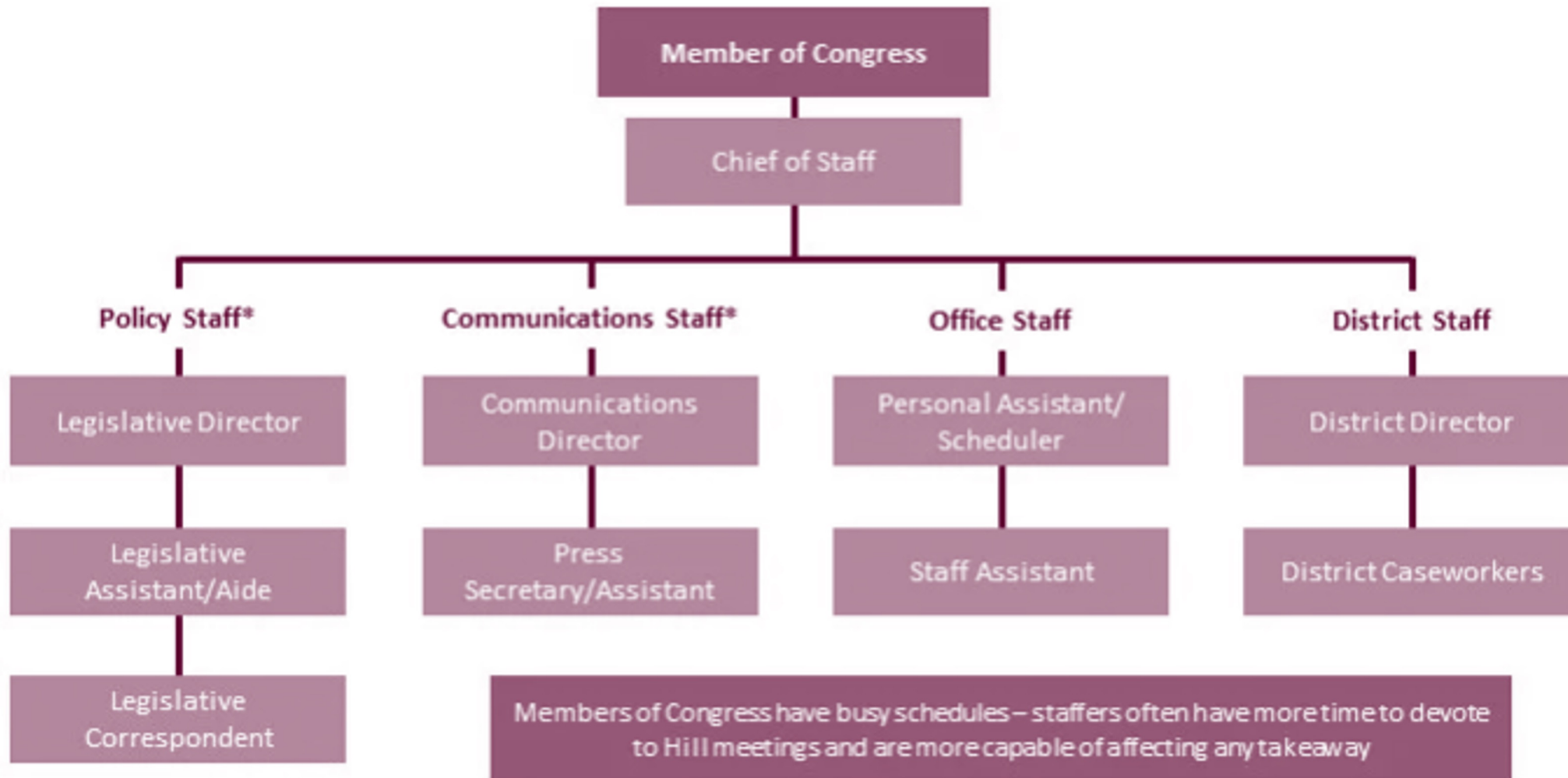
- Maximize exposure to broad array of Congressional offices
- Prioritize key members of Congress (e.g., Committees of jurisdiction)

Schedules will be distributed when you check-in on July 15th

Meetings are scheduled from 9am – 4pm and it is critical that you **attend all confirmed meetings**



# Typical Organizational Structure of a Congressional Office



\*most meetings will be with policy staff, as members of Congress will be home in their districts over recess



## Suggested Meeting Flow

1. Introduce everyone
2. State the issues
3. A few participants share personal stories or specific examples of how an issue has impacted them
4. Inquire about the Senator/Representative's position/seek feedback from the staffer
5. Make the "Ask"
6. Thank the staffer for their time & offer to have ARM follow-up

\*Teams should determine speaking roles and meeting run-of-show during Congressional Team huddle





# **Advocacy Best Practices**

# Advice from the Experts

## Jordan LaCrosse

Vice President, Avenue Solutions

## Miranda Franko

Senior Policy Advisor, Holland & Knight

## Dan Farmer

Principal, BGR Group





# Tips for an Effective Meeting

## Do

- Arrive on time and prepared.
- Greet the Member and/or staff with a handshake and formally introduce yourself (and all others present).
- Obtain information about their depth of knowledge and understanding of occupational therapy.
- Present your information in a direct, organized, and timely manner.
- Cite specific bills, titles, and issues to ensure clarity.
- Relate the information directly to the legislator's constituents using personal stories, which helps translate the issues into tangible, realistic problems.
- Demonstrate why this issue is important to you and your Members of Congress.
- Limit the length of presentation to preserve time for discussion – “talk with” not “talk at”.
- Be attentive to the legislators' positions, comments, and feedback.
- Share critical information with ARM team to ensure accurate follow up

## Don't

- Get discouraged if your legislator's staff arrive late for your meeting.
- Assume the legislator knows about the field of cell and gene therapy.
- Assume that the legislator is familiar with your issue(s). Thousands of bills are introduced in each Congress, and legislators and staff will not be able to remember them all.
- Discuss numerous bills or address unrelated issues.
- Maintain a narrow-minded perspective. Be attentive and open to different views and feedback.
- Demonstrate angry, threatening, or confrontational behavior. Leave the office with a positive feeling of cell and gene therapy and desire for future collaboration.
- Treat the meeting as a one-time event. Develop a working relationship with your legislator.





# Q&A

## Need Help?

For **policy questions** contact Erica Cischke [ecischke@alliancerm.org](mailto:ecischke@alliancerm.org)

For **schedule questions** contact Crimson Consulting [lindsey@crimson1.com](mailto:lindsey@crimson1.com)

For **hotel and transportation questions** contact Brittany Miller  
[bmiller@alliancerm.org](mailto:bmiller@alliancerm.org)

**Board members** should contact Tommy Szabo [tszabo@alliancerm.org](mailto:tszabo@alliancerm.org) with any questions about the Board of Directors meeting or other Board engagements

