

# Understanding Statewide Implementation of New Disorders

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Presented to the Advisory Committee on Heritable Disorders in  
Newborns and Children

April 23, 2019

*This project is supported by the Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) as part of an award totaling \$4,000,000 with 0% financed with non-governmental sources. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by HRSA, HHS, or the U.S. Government. For more information, please visit [HRSA.gov](https://www.hrsa.gov)*

# Overview

## New Disorders Recently Added to the RUSP

<b>New Disorder</b>	<b>Date added</b>
<b>Pompe</b>	March 2, 2015
<b>MPS I</b>	February 16, 2016
<b>X-ALD</b>	February 16, 2016
<b>SMA</b>	July 2, 2018

# Readiness Tool Phases

Phase 1 – Approval/Authority to Screen

Phase 2 – Lab and Follow-Up Logistics

Phase 3 – Education

Phase 4 - Implementation

# Research Questions

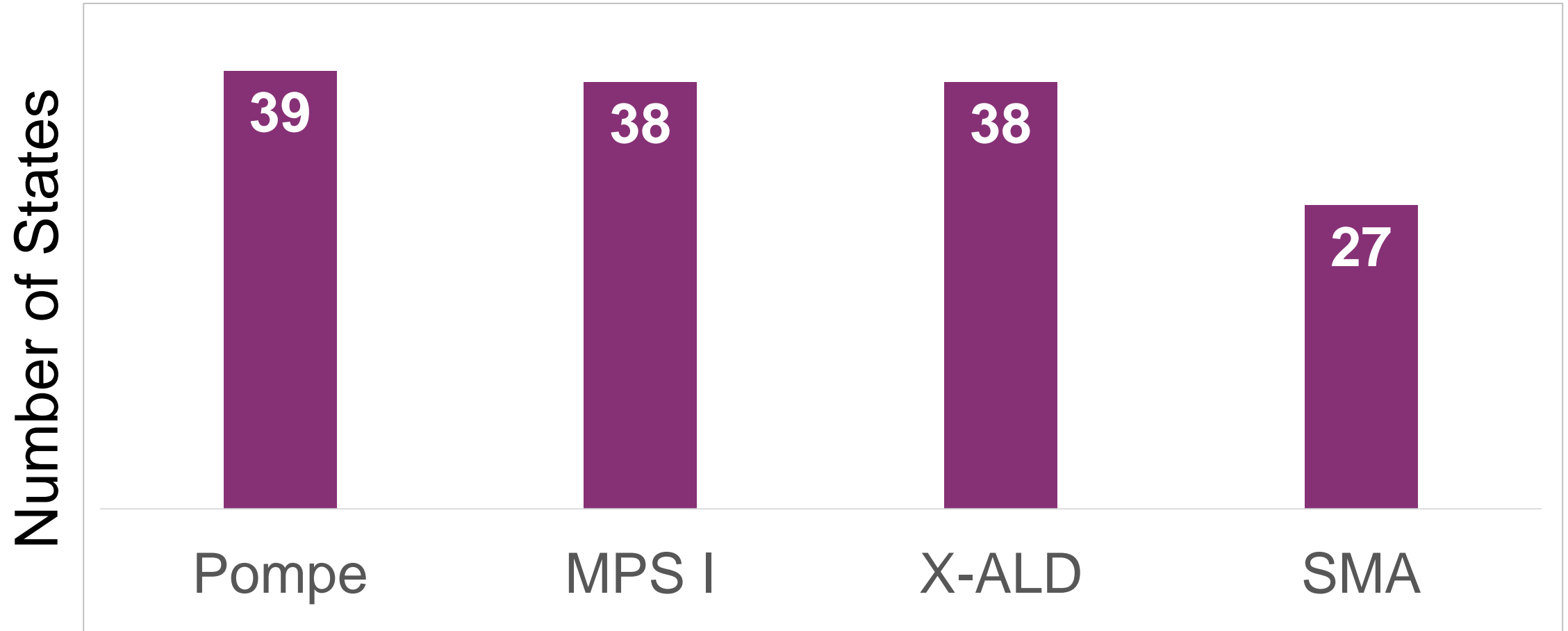
1. How long does it take to implement statewide screening for a new disorder?
  - a. How long does each readiness phase take?
  - b. Where is the most time spent?
  
2. What are the facilitators and challenges for statewide screening?

# Background & Methods

# Methods to Assess Time

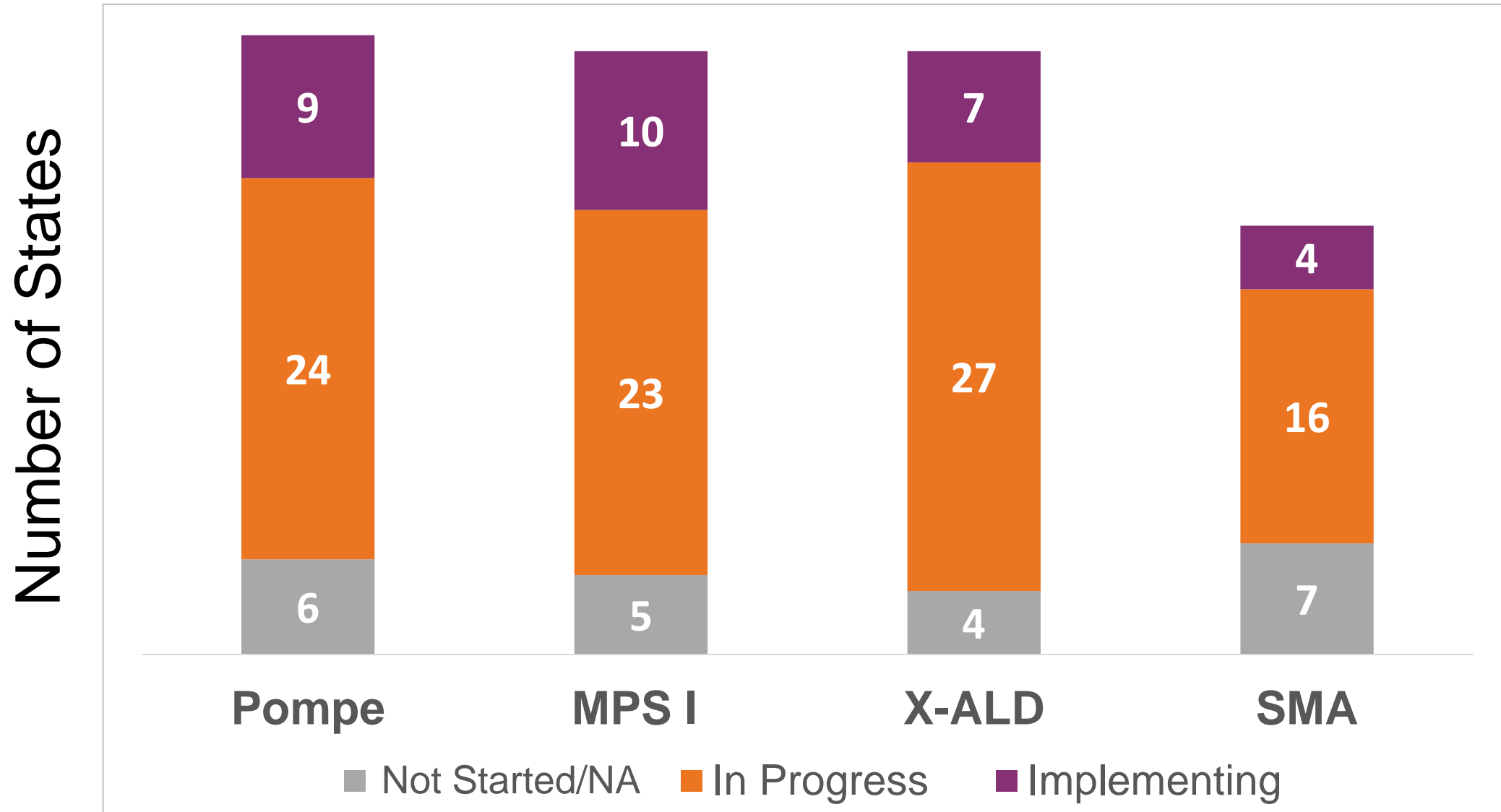
- **Readiness Tool (N=39)**
  - 16 New Disorder awardees (NewSTEPs/APHL)
  - 2 Peer Resource Networks
  - 21 states who attended New Disorder annual meetings, but did not receive funding

# Readiness Tool Completion





# Implementation Status as of February 28, 2019



# Many States are in still In-Progress

Disorder	N	Screening Implemented	In Progress <i>(completed at least one activity)</i>	Not Started <i>(has not initiated any activities)</i>
<b>Pompe</b>	39	9 (23.1%)	<b>21 (53.8%)</b>	6 (15.0%)
<b>MPS I</b>	38	10 (26.3%)	<b>19 (50.0%)</b>	5 (13.2%)
<b>X-ALD</b>	38	7 (18.4%)	<b>22 (57.9%)</b>	4 (10.5%)

# Methods for Identifying Barriers and Facilitators

- **New Disorder Annual Reports (N=16)**
  - Barriers or Facilitators are only included in this presentation if mentioned by at least 3 awardees
- **Key Informant Interviews (N=7)**
  - Barriers or Facilitators are only included in this presentation if mentioned by at least 2 awardees

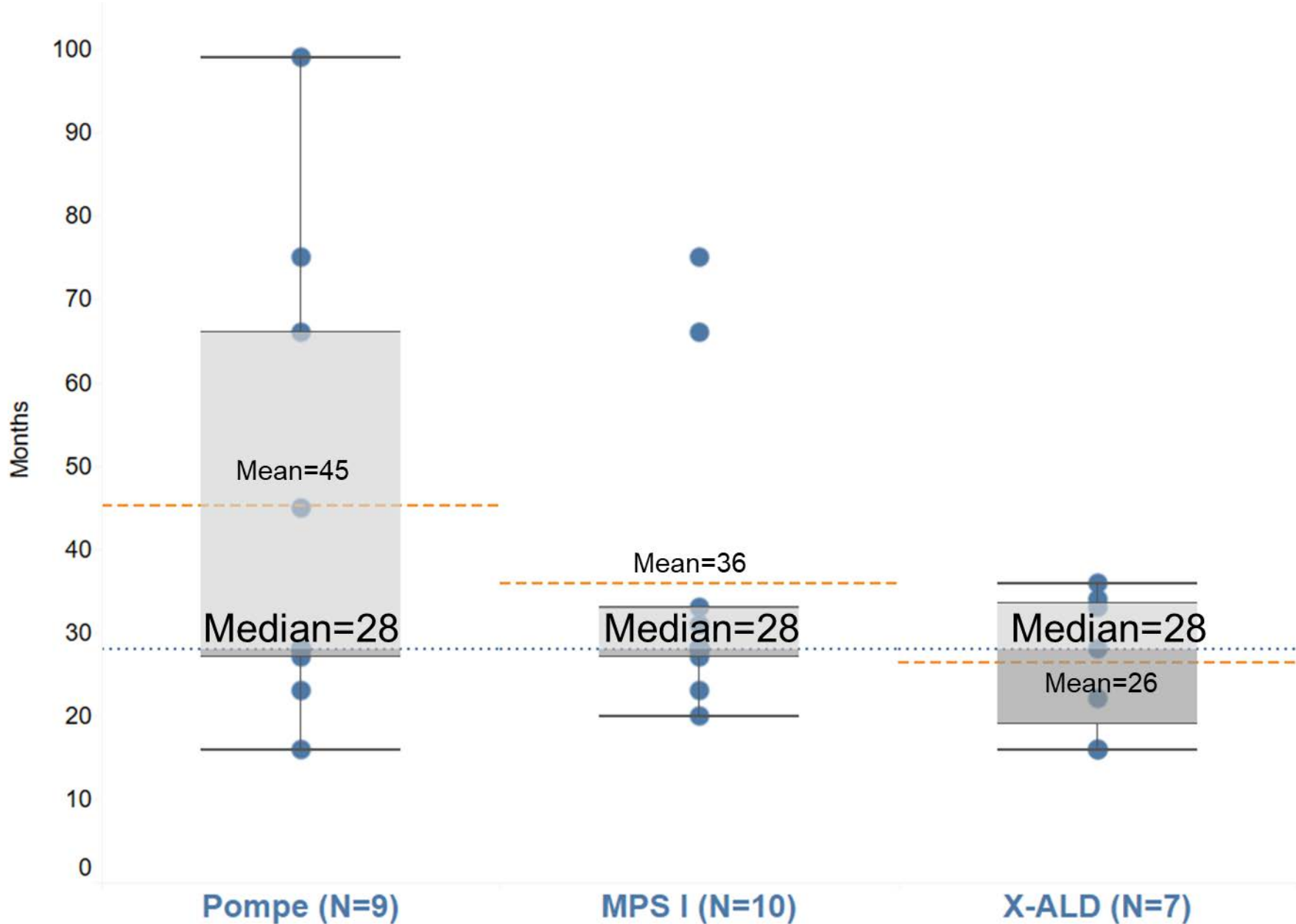
# Limitations

- Last Collection of Readiness Tool data was February 28, 2019.
- Some states provided a time estimate versus actual dates
- Not all states who are currently screening for new disorder completed/updated the Readiness Tool
- Collected facilitators and barriers only from participating programs; does not include all states

**How long does it  
take to implement  
statewide  
screening for a  
new disorder?**



# Time From First Activity to Statewide Screening



**All 9** NBS programs that implemented statewide screening for Pompe also implemented for MPS I

**4** participating programs implemented statewide screening for Pompe, MPS I, **and** X-ALD

*Data from Readiness Tool (N=39)*

*“Our timelines are **longer** because we were the **first** [program] and had so much to **validate** before we could start our full-population pilot.”*



**Gaining assistance from other states**  
was a facilitator to implementing  
screening statewide



Nine states said Collaboration between states makes it easier to implement statewide screening for new disorders



Peer-Network Resource Centers was also mentioned as a Facilitator by 9 states



Three states said a barrier to implementing statewide screening was limited information from other NBS labs to provide knowledge and experiences

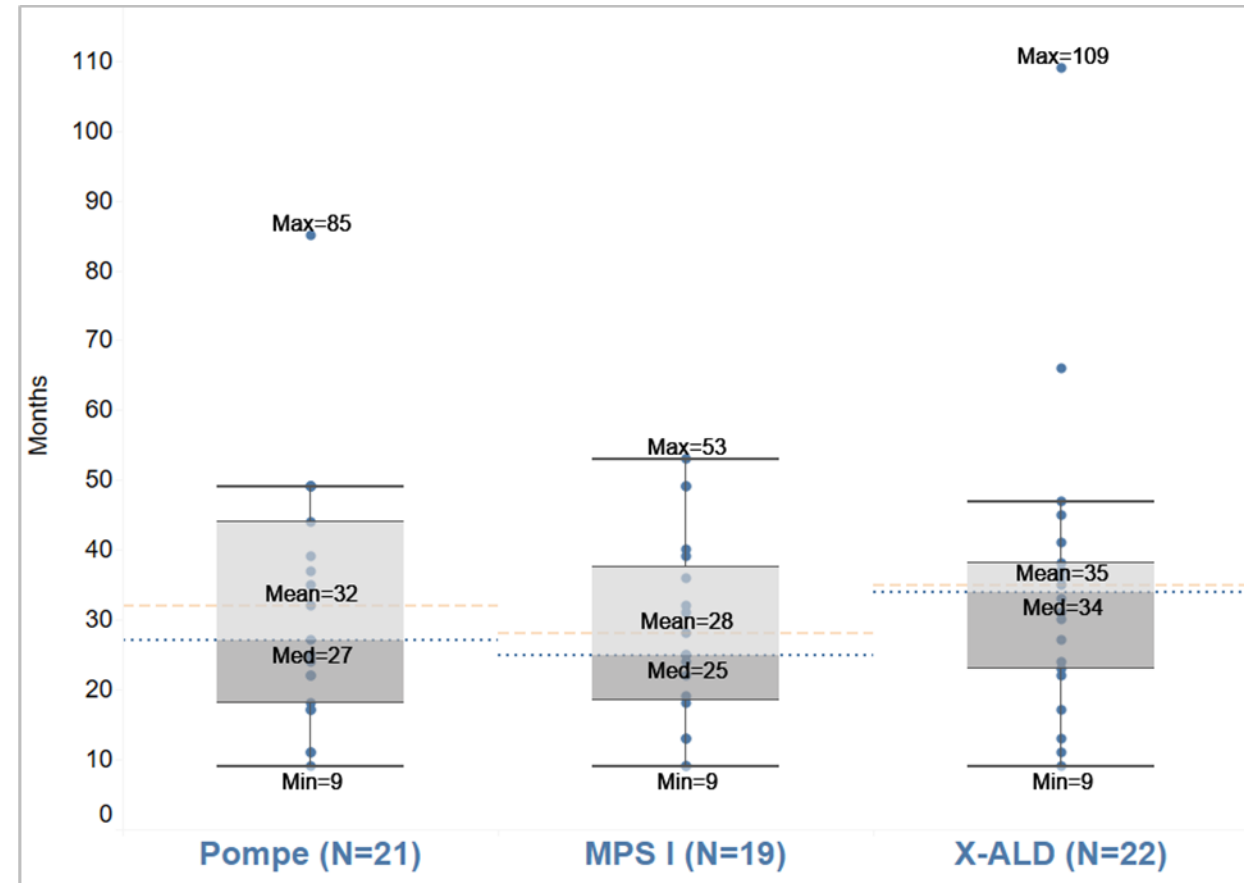
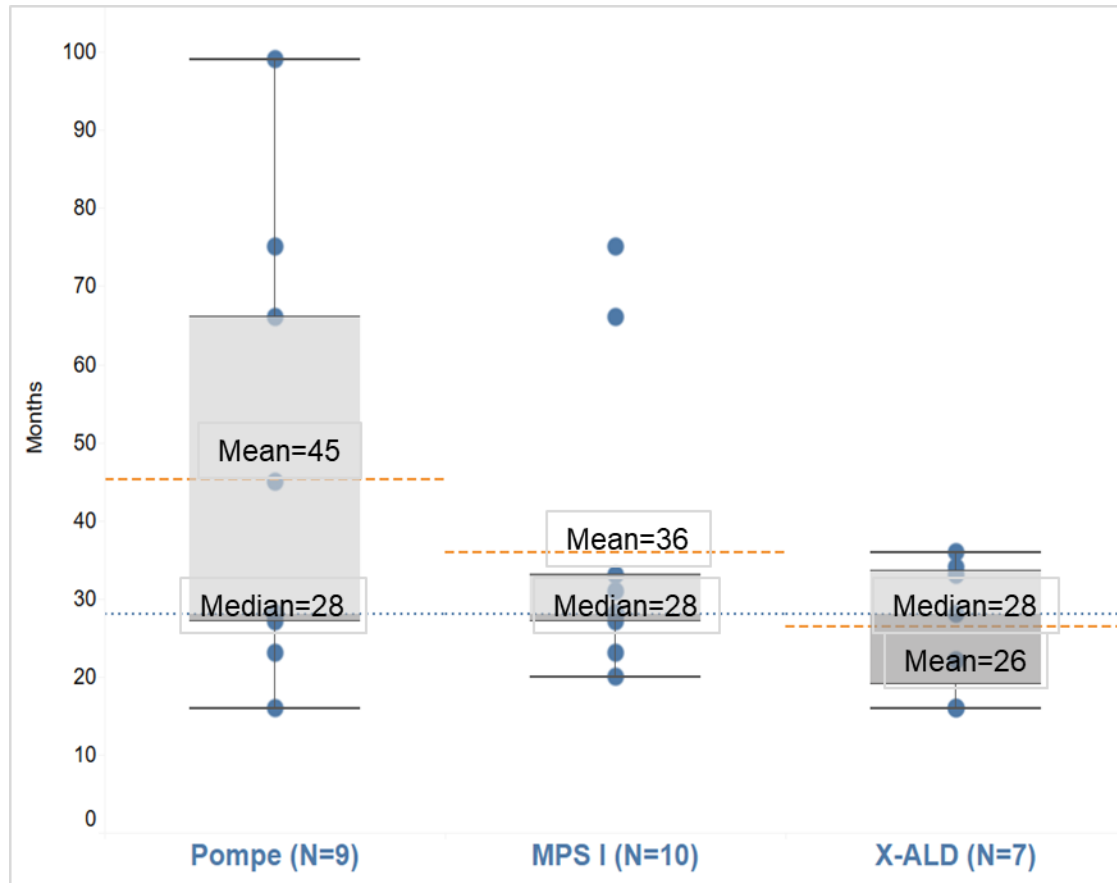


**How does  
implementation time  
compare for states that  
are screening vs. those  
that are in progress?**

# Time to Statewide Screening (Implemented)

vs.

# Time Spent working Towards Screening (In Progress)



**How Long  
Does Each  
Readiness  
Phase Take?**



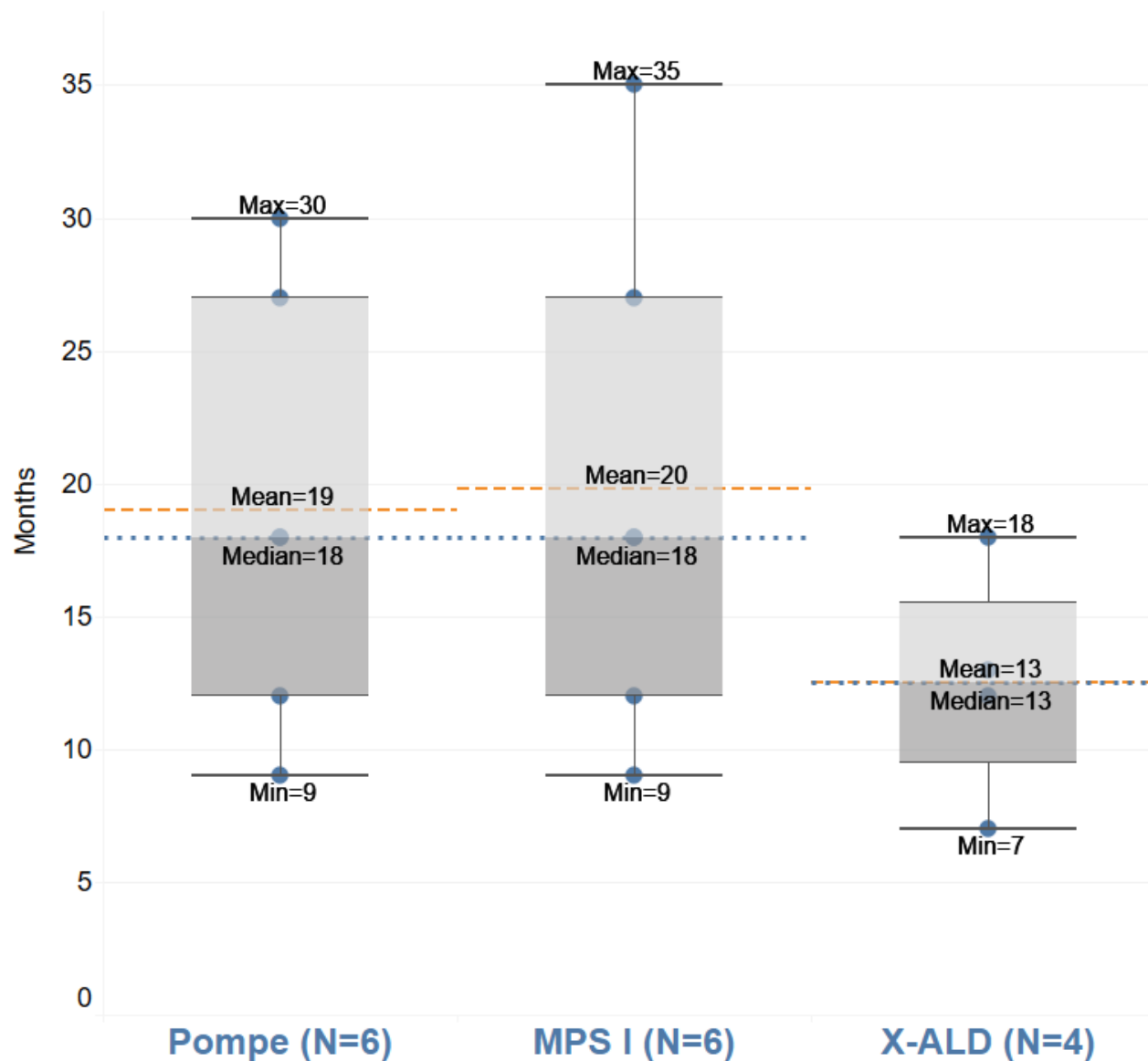
# Phase 1 – Authority to Screen

**25** participating states (64%) received approval to screen for the new disorder from at least one state group

**17** participating states (44%) received approval for funding to screen for the new disorder

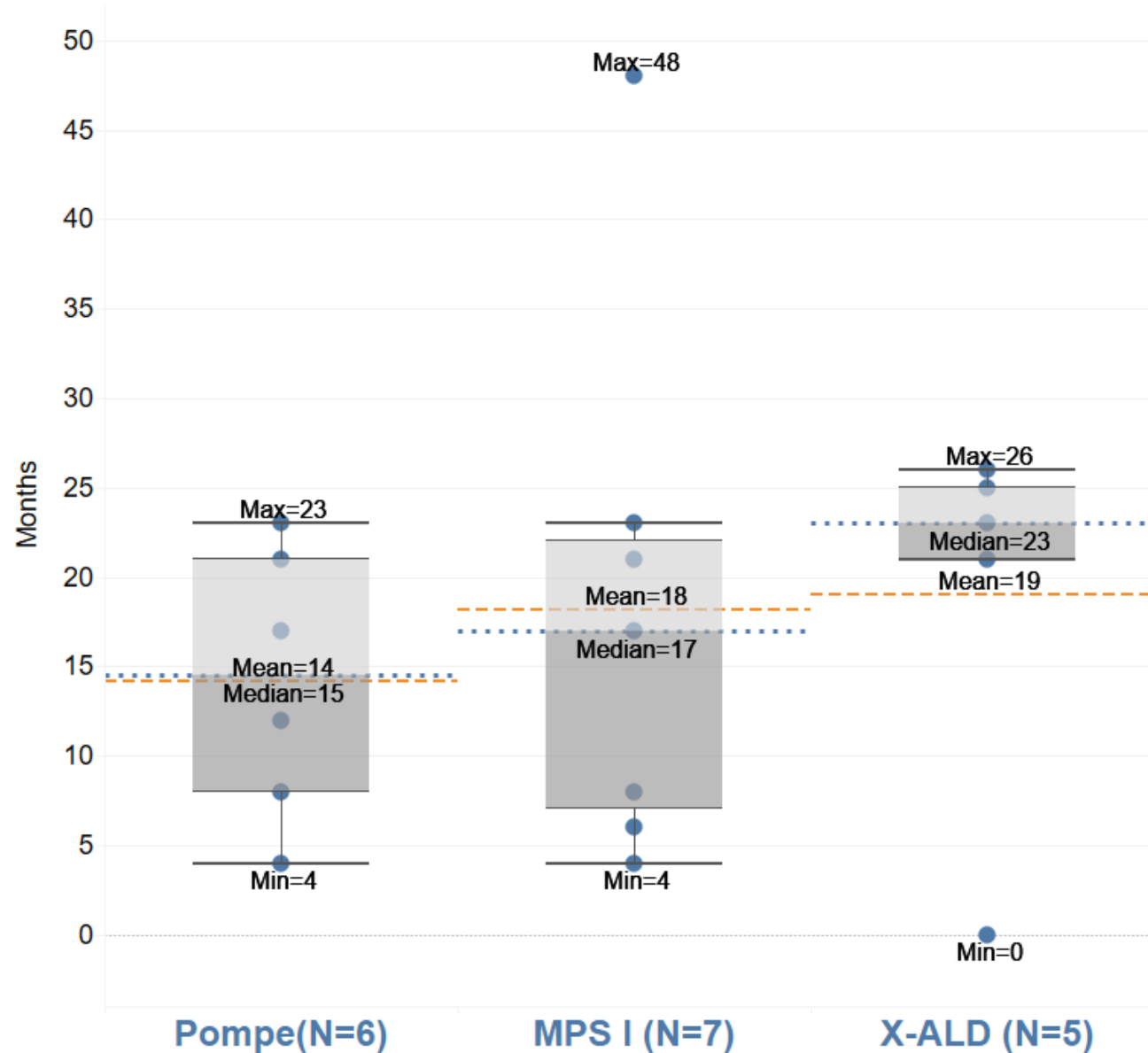
# Phase 1a–Approval/Authority to Screen

## *For Programs that Implemented Statewide Screening*



# Phase 1b—Approval for Funding

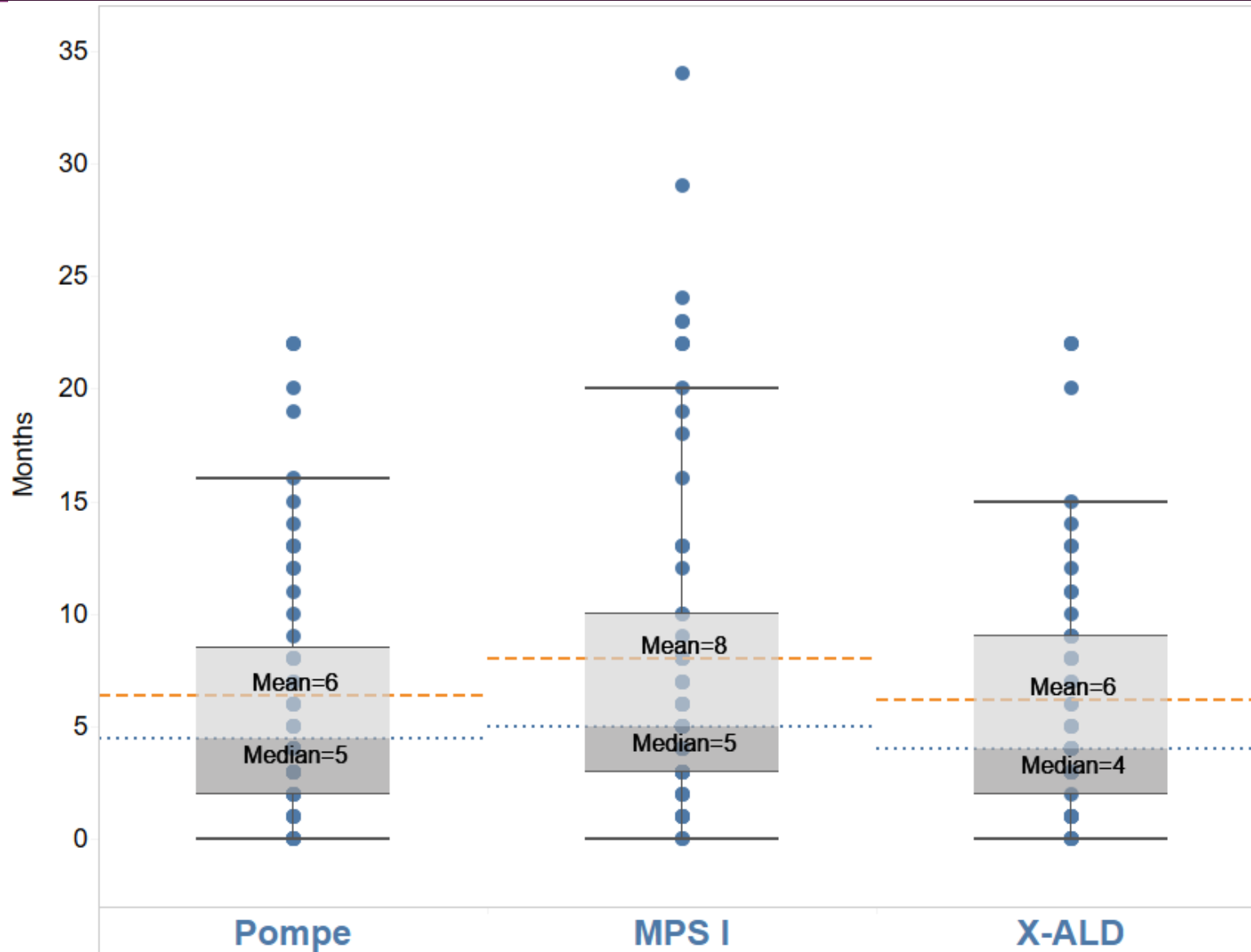
## *For Programs that Implemented Statewide Screening*





# Phase 1—Approval/Authority to Screen

## *Time to Complete Each Activity in Phase*



# Phase 1-Approval/Authority to Screen *The Most Time Intensive Steps*

Obtaining approval from the state NBS Advisory Committee took a median of **6 months**

Obtaining approval from State Budget Authority took a median of **6 months**

Developing a budget took a median of **5 months**

*Data from Readiness Tool (N=28)*

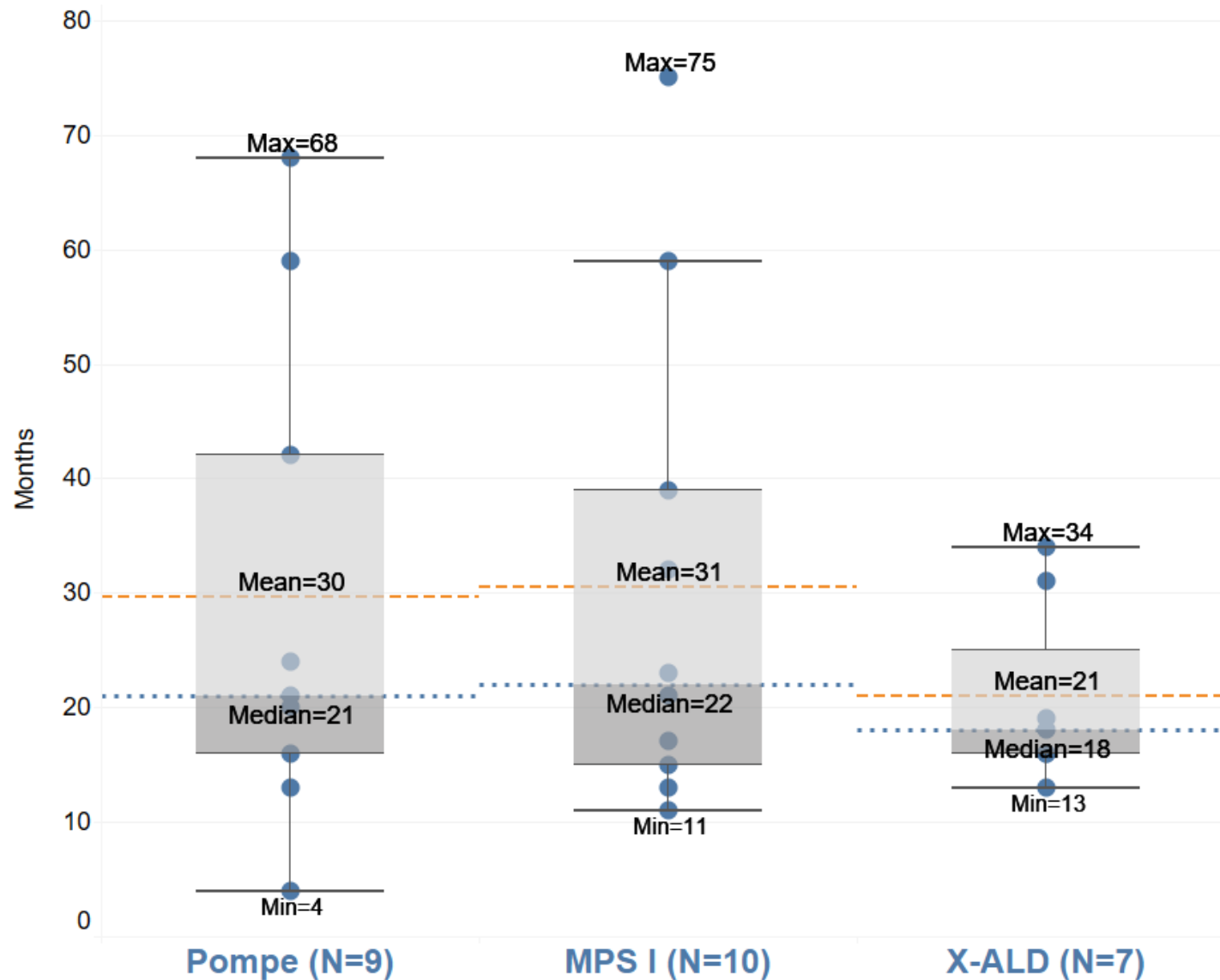
*“In [our] administrative code we review all new disorders that come onto the RUSP and report back to the full-advisory committee. That [state-based] committee will vote on recommendation . . . and send to the Commissioner of Health. [The] Commissioner will then take to the Board of Health and say [we] want to change regulations. As soon as we get the Commissioner of Health [to agree], that starts a process where you post notice of intent to change regulations, 30 days of comments, then edit notes based on public comment, go to planning and budget, attorney general, etc. and each has to sign off. Each [approval step] could take 30 to 60 days. **All those steps take about a year to 18 months for regulations to pass.** [This process] gives us time to systematically and carefully bring up a disorder.”*

*“In [our] administrative code we review all new disorders that come onto the RUSP and report back to the full-advisory committee. That [state-based] committee will vote on recommendation . .and send to the Commissioner of Health. [The]Commissioner will then take to the Board of Health and say [we] want to change regulations. As soon as we get the Commissioner of Health [to agree], that starts a process where you post notice of intent to change regulations, 30 days of comments, then edit notes based on public comment, go to planning and budget, attorney general, etc. and each has to sign off. Each [approval step] could take 30 to 60 days. **All those steps take about a year to 18 months for regulations to pass. [This process] gives us time to systematically and carefully bring up a disorder.**”* -Key Informant Interview



# Phase 2 – Laboratory Readiness

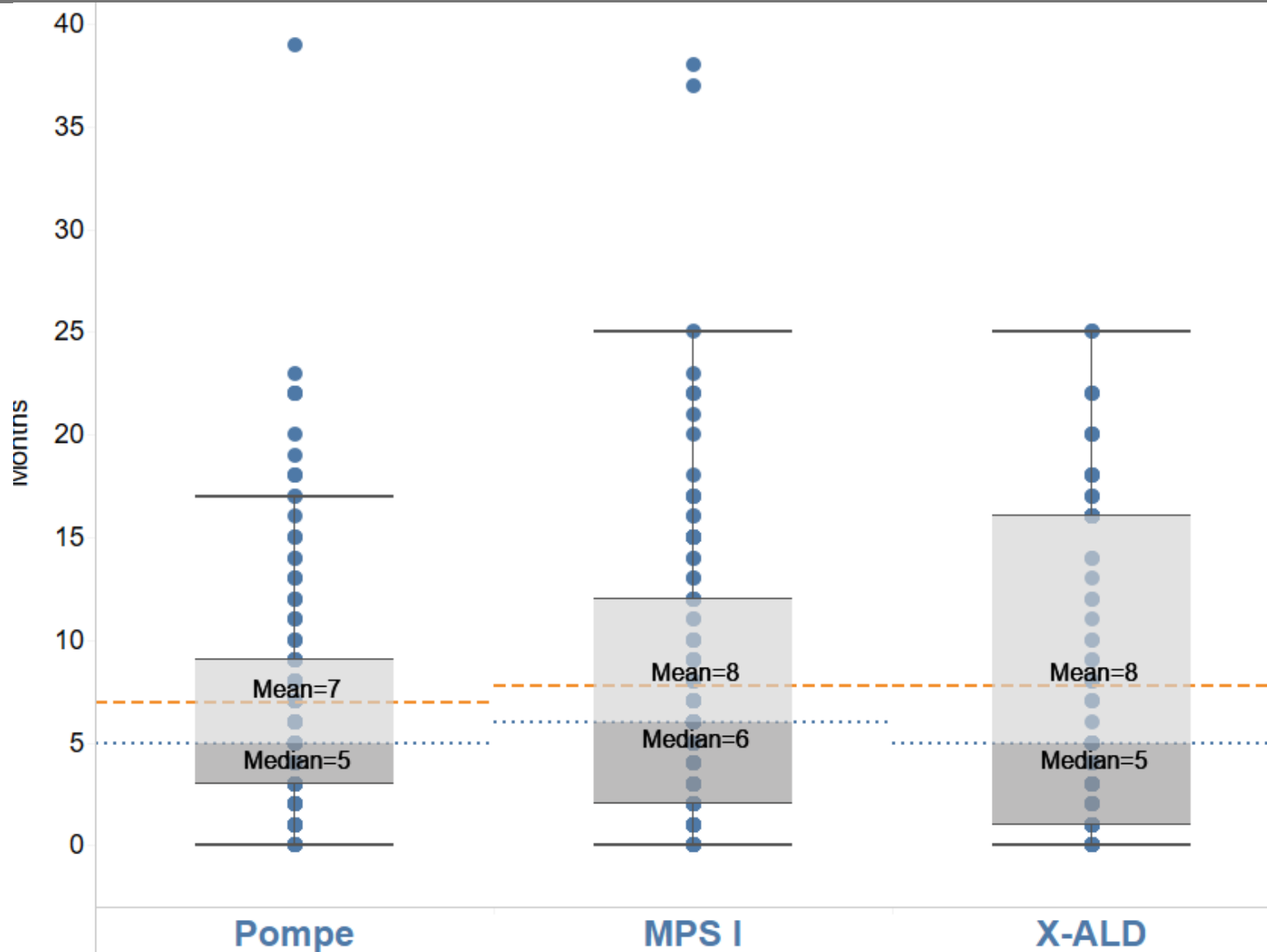
## *For Programs that Implemented Statewide Screening*



**23** participating states (59%) completed at least one Lab Readiness Activity

# Phase 2 – Laboratory Readiness

## *Time to Complete Each Activity in Phase*





# Phase 2 – Laboratory Readiness

## *Most Time Intensive Steps*

Median time of **12 months** to identify laboratory space, modify, and install equipment

Median time of **9 months** to identify needed equipment.

Median time of **9 months** to develop a lab staffing plan.

Median time of **9 months** to train lab staff.

*Data from Readiness Tool (N=23)*

**Staffing** was both a barrier and a top facilitators for being able to implement screening statewide



Laboratory-specific staffing shortage was mentioned by 9 states.



Ability to hire lab staff was mentioned as facilitator by 3 states.

**Equipment** was both a barrier and a facilitator for being able to implement screening statewide



Ability to get needed equipment and assays was mentioned by 6 states as a facilitator



Inability to get equipment or not having access to needed equipment was mentioned as a barrier by 6 states



Inability to get equipment up and running was listed as a barrier by 3 states



Not having an FDA approved kit and/or instrumentation was a barrier for 3 states



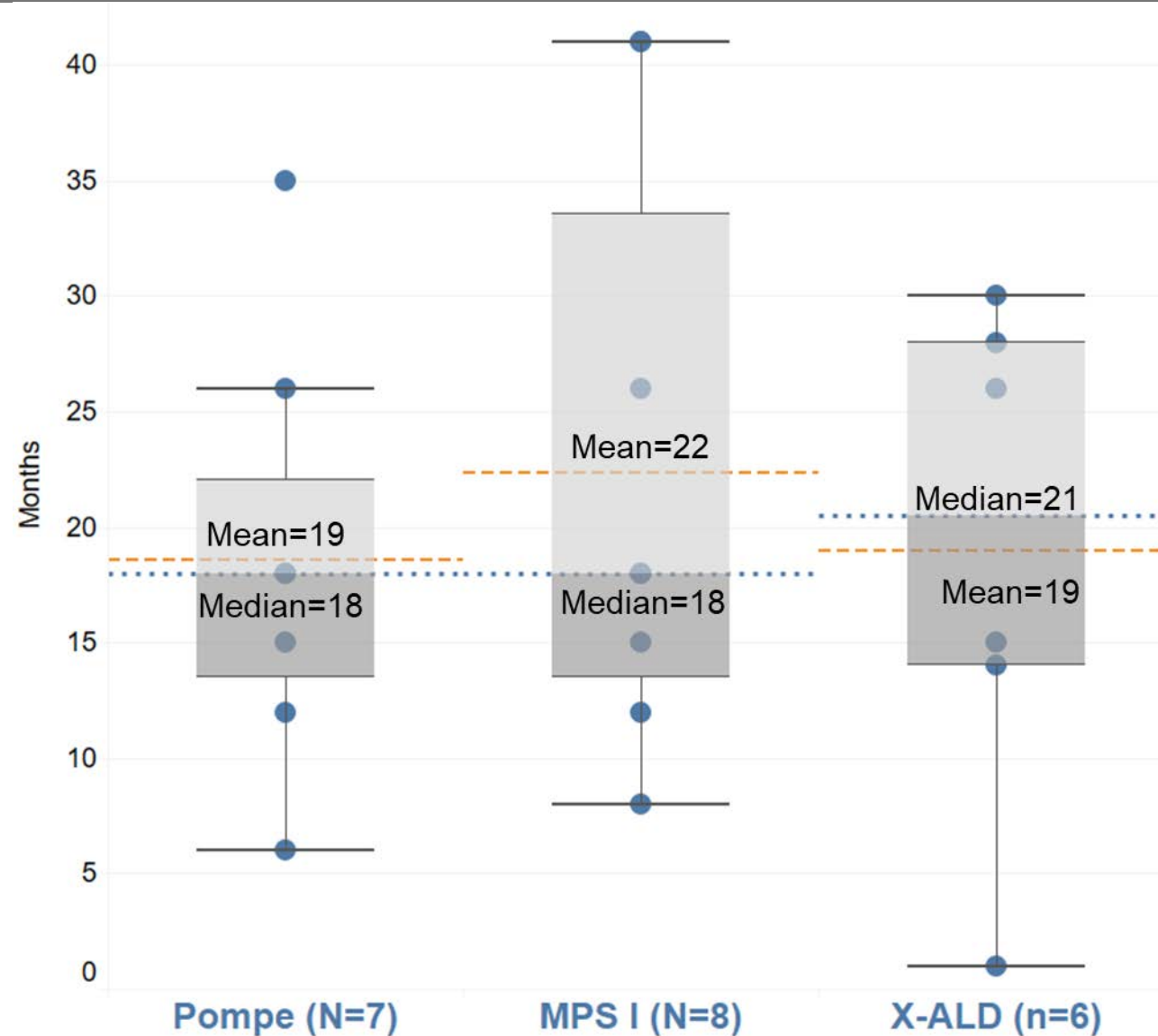
Validation of methodology was identified as a facilitator by 4 states



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# Phase 2 – Follow-Up Readiness

## *For Programs that Implemented Statewide Screening*

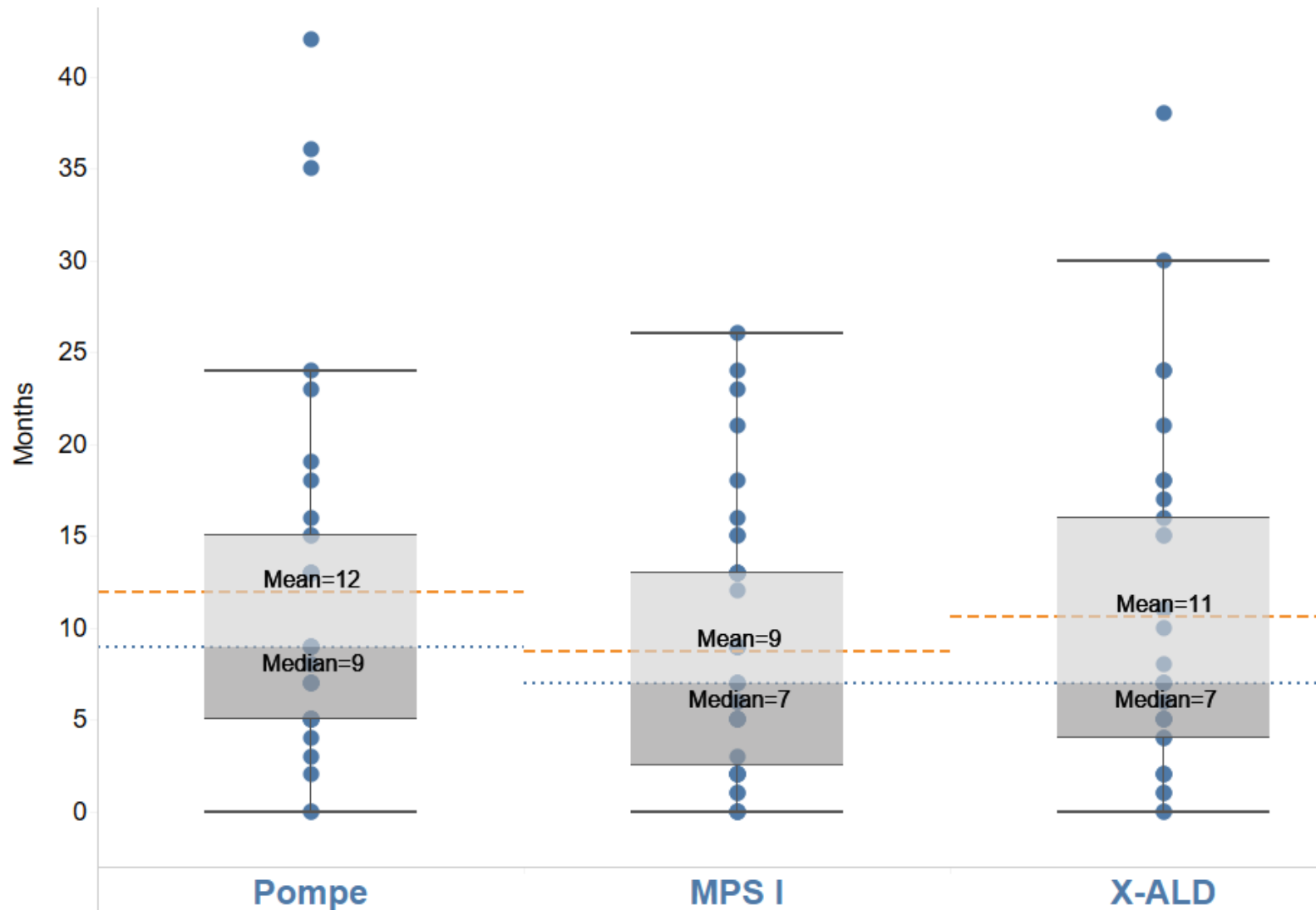




**20** participating states (51%) completed at least one Follow-Up Readiness Activity

# Phase 2 – Follow-Up Readiness

## *Time to Complete Each Activity in Phase*



# Phase 2 – Follow-Up Readiness

## *Most Time Intensive Steps*

**10.5 months** median time to identify medical specialists or treatment centers

**9 months** median time to develop and gain buy-in for short-term

**9 months** median time to develop and gain buy-in for long-term follow-up protocols

*Data from Readiness Tool (N=20)*

**Staffing** was both a barrier and a top facilitator for being able to implement screening statewide



Follow-Up staffing shortage was mentioned by 3 states



Ability to hire follow-up staff was mentioned as a facilitator in 9 states



Setting up follow-up protocols was also identified as a facilitator by 5 states



Difficulty around establishing long-term follow-up protocols were mentioned as a barrier to implementation in 3 states



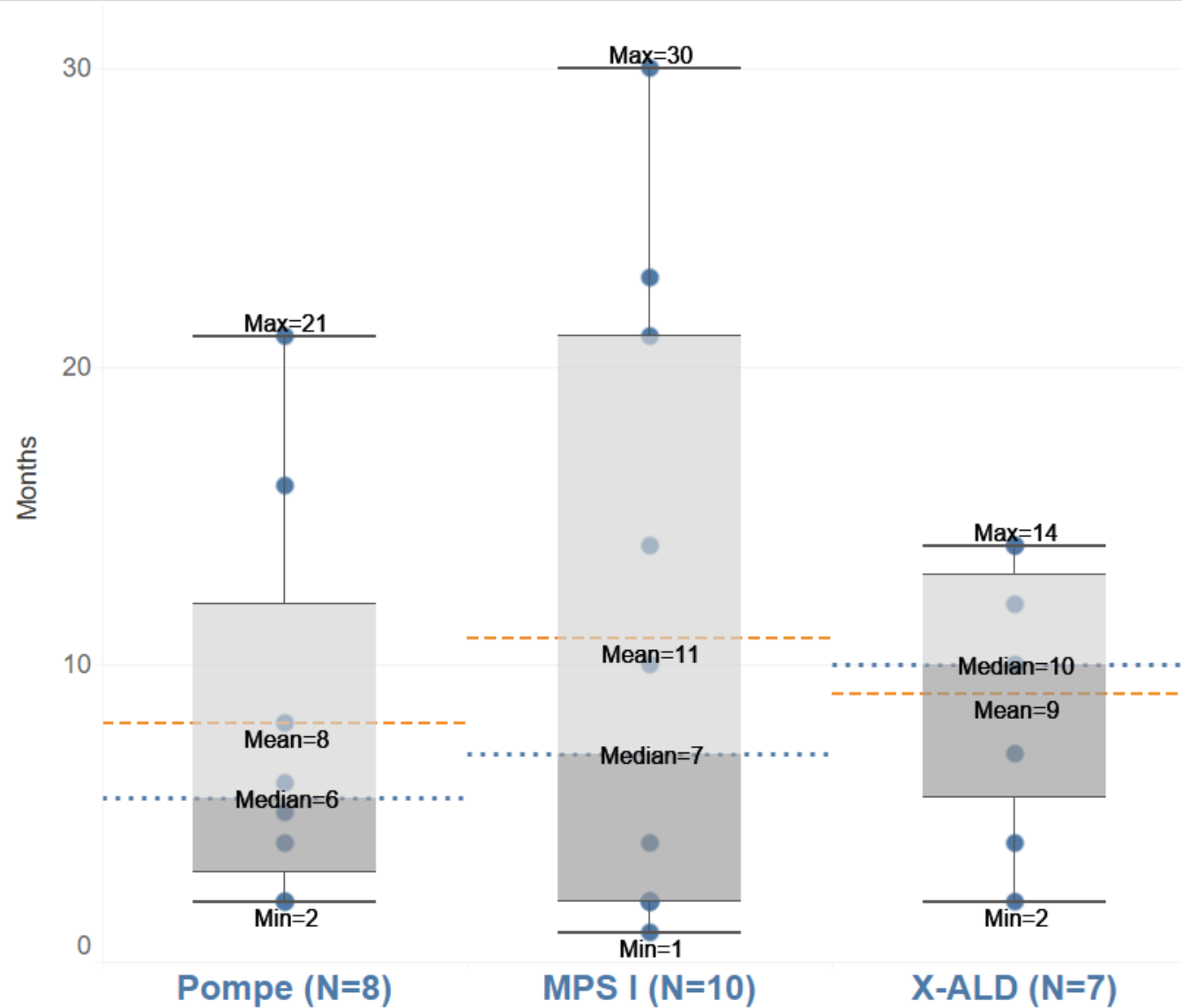
# Phase 2 – IT Readiness

***IT was the most frequently listed activity that started after implementation***



# Phase 2 – IT Readiness

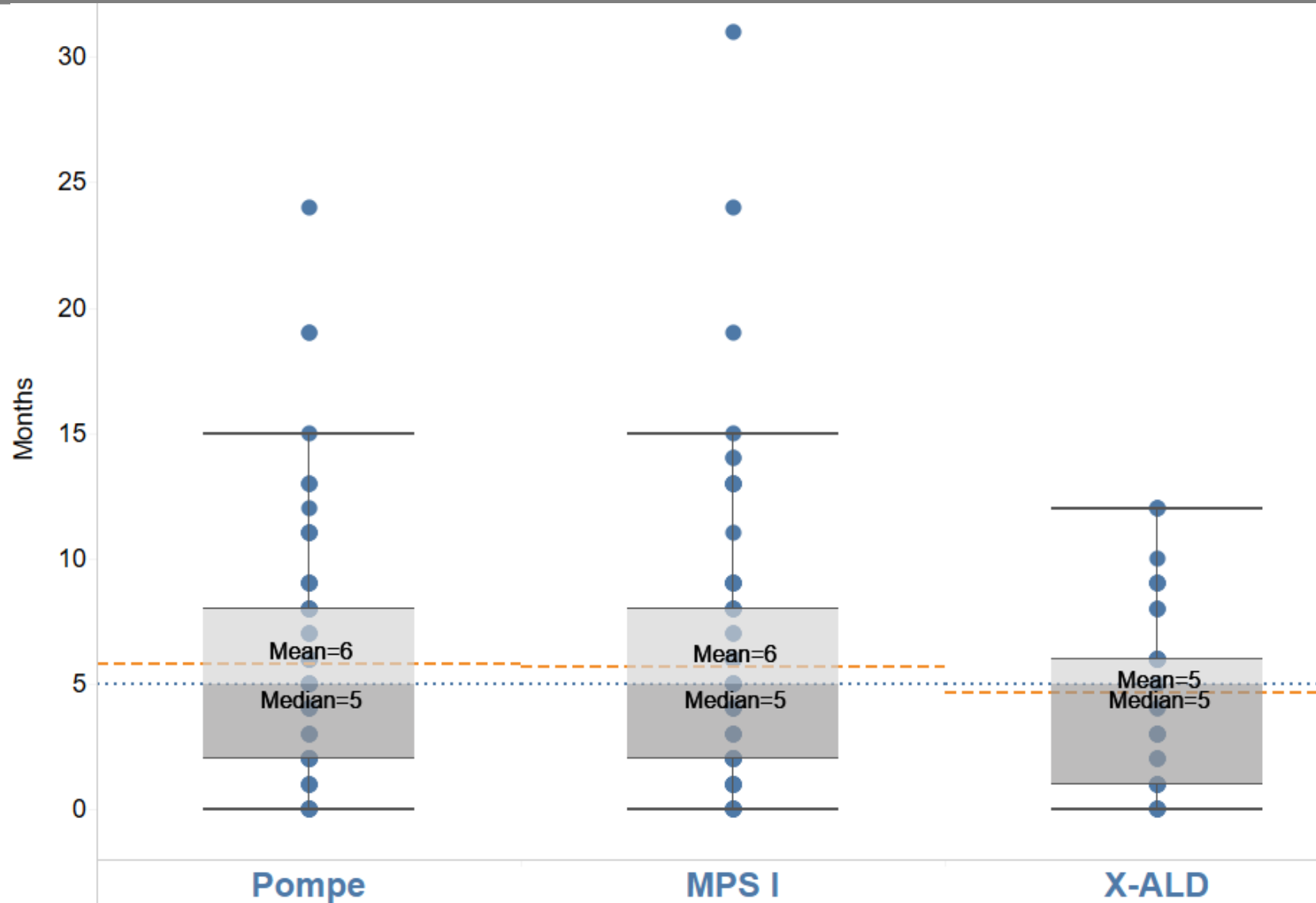
## *For Programs that Implemented Statewide Screening*



**20** participating states (51%) completed at least one IT Readiness Activity

# Phase 2 – IT Readiness

## *Time to Complete Each Activity In Phase*



# Phase 2 – IT Readiness

## *Most Time Intensive Step*

Median time of **8 months** to  
describe and develop  
specifications for LIMs

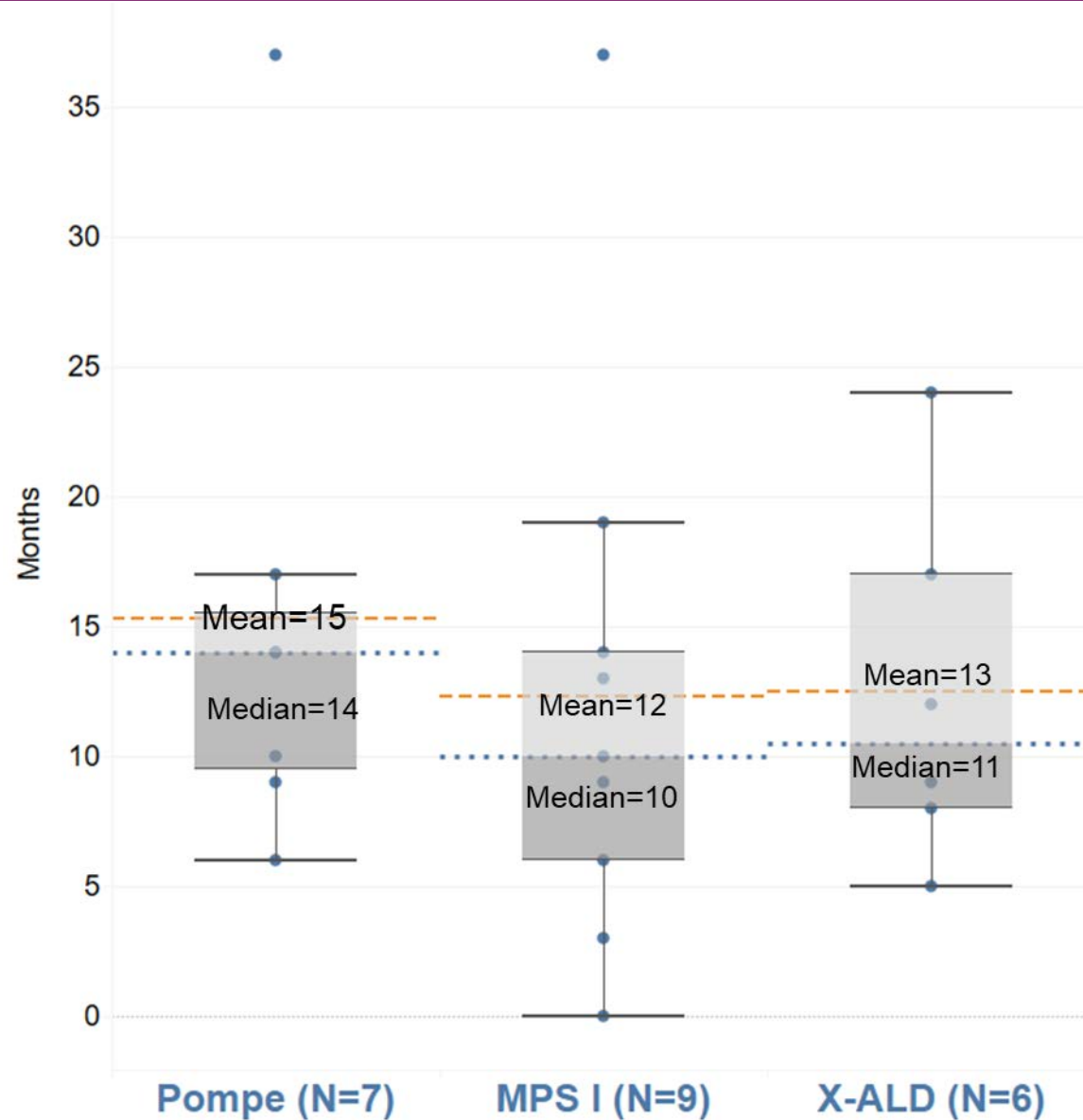
*Data from Readiness Tool (N=20)*



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# Phase 3 – Education Readiness

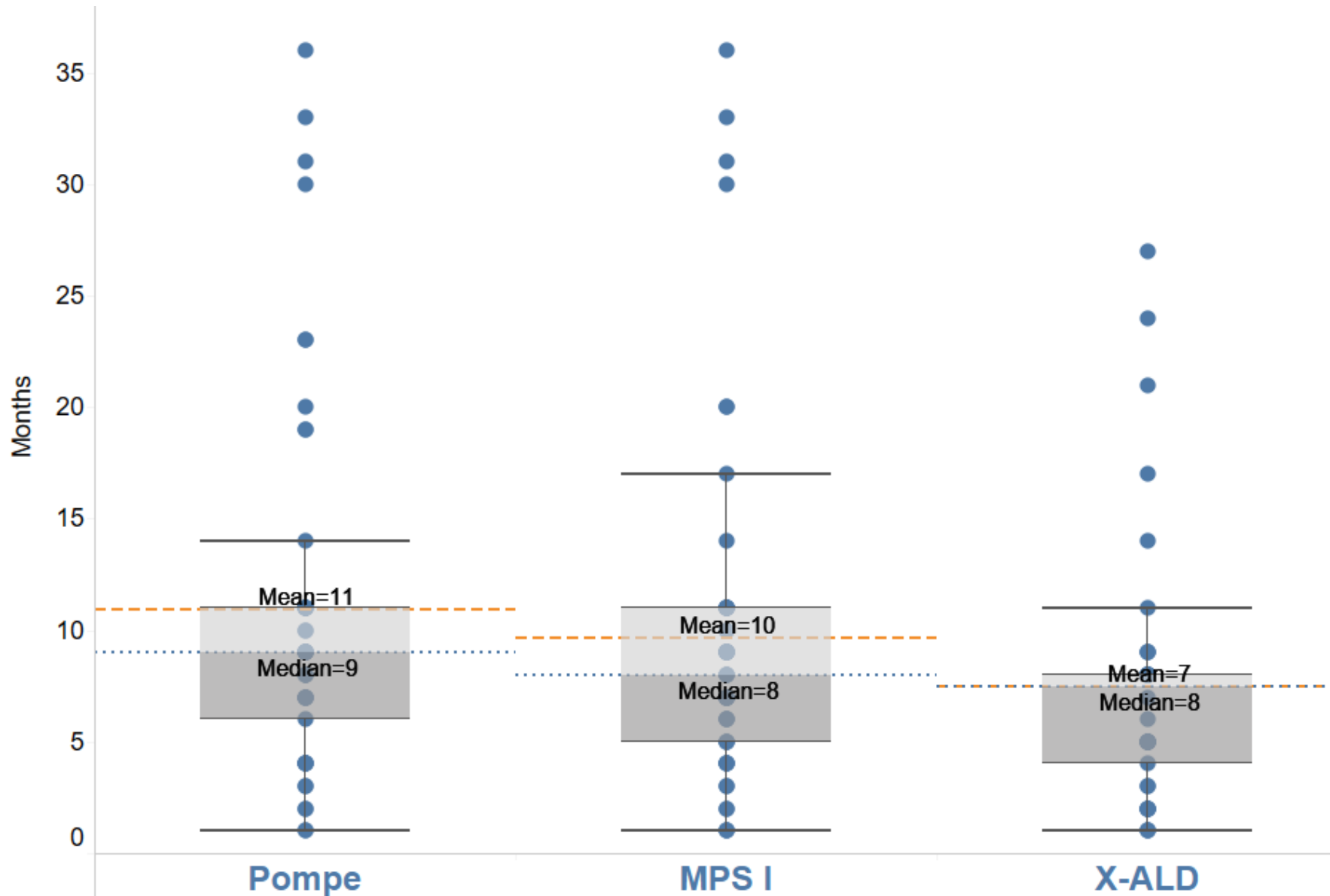
## *For Programs that Implemented Statewide Screening*



**16** participating states (41%) completed at least one Education Readiness Activity

# *Phase 3 – Education Readiness*

## *Time to Complete Each Activity in Phase*





# Phase 3 – Education Readiness

## *Most Time Intensive Steps*

**9 months** median time initiate an education strategy for family and general public education materials

**9 months** median time to identify and modify education materials for the general public

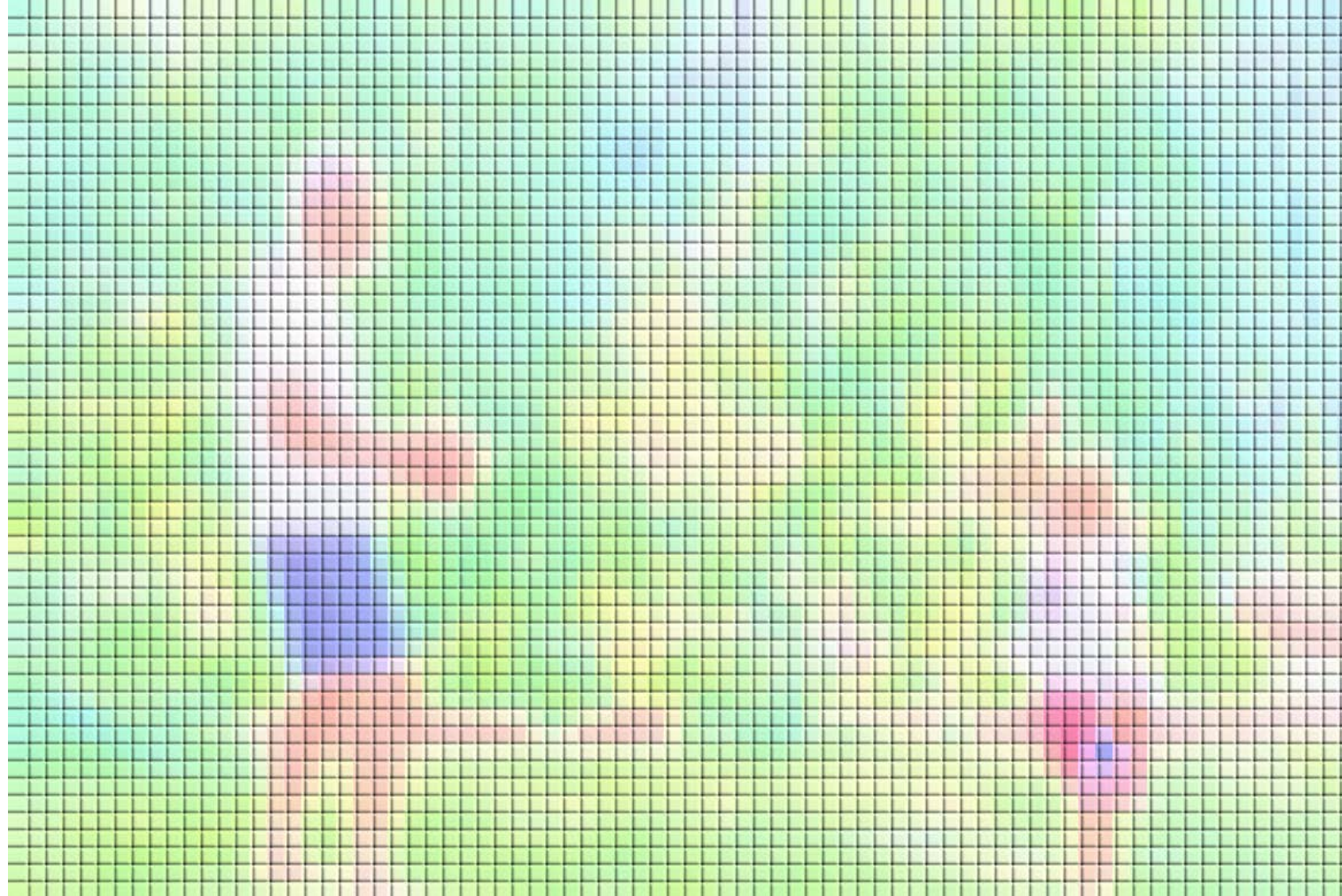
**9 months** median time to identify or create measures to track impact of provider education materials

*Data from Readiness Tool (N=16)*



Input from various stakeholders on education was identified as a facilitator by 7 states

# Final Thoughts



# Thank You To All Those Involved With This Project

- Kshea Hale
- Sarah McKasson
- Marci Sontag
- Sikha Singh
- Jelili Ojodu
- Joshua Miller
- Guisou Zarbalian (for HIA)
- All the states who provided and updated the Readiness Tool
- The NewSTEPs Steering Committee
- All NBS experts who helped finalize the readiness tool



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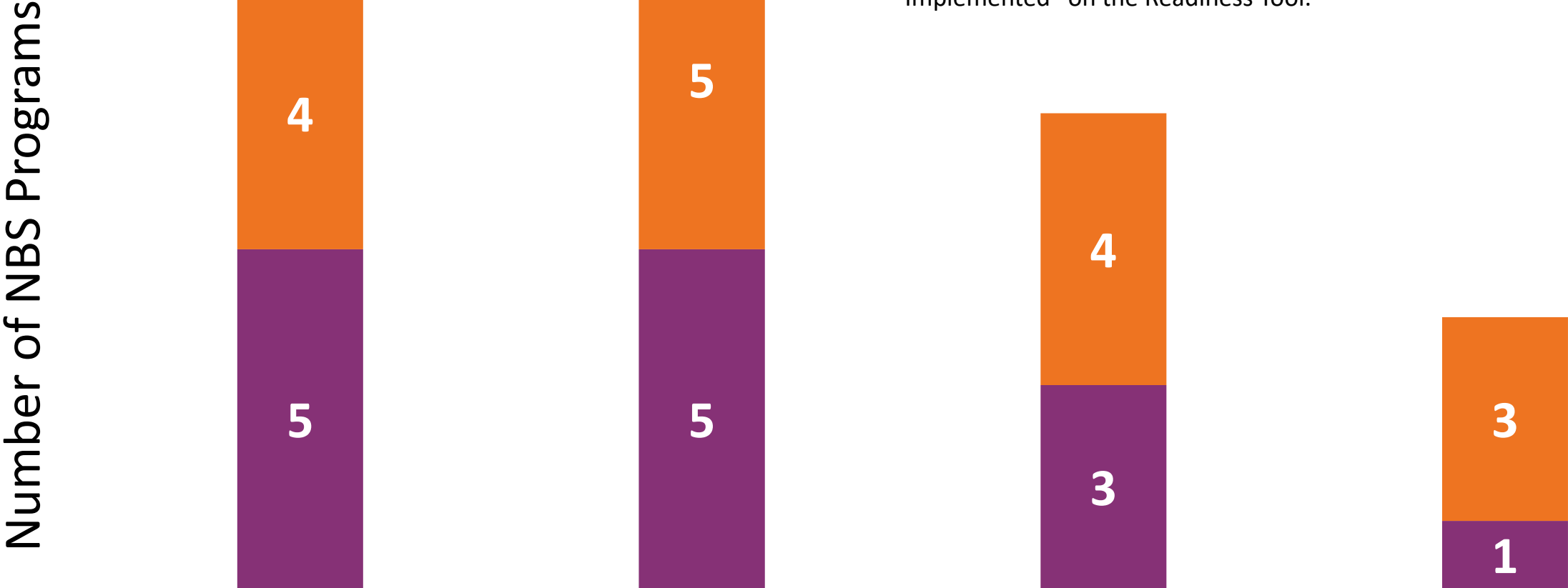
**Not Covered In Presentation But May Help  
with Questions**

***Approval for Screening*** and/or ***Funding***, while is often the first step, it is not always the first step.....

***Ensuring Adequate Space for Testing*** was the second most frequently listed first activity on the Readiness Tool

# Directed to Screen by State Legislation for those States Who Provided Readiness Tool Data and Implemented Statewide Screening

Note: This only includes information provided on the Readiness Tool February 28, 2019. Count is based on the number of states who checked "Implemented" on the Readiness Tool.





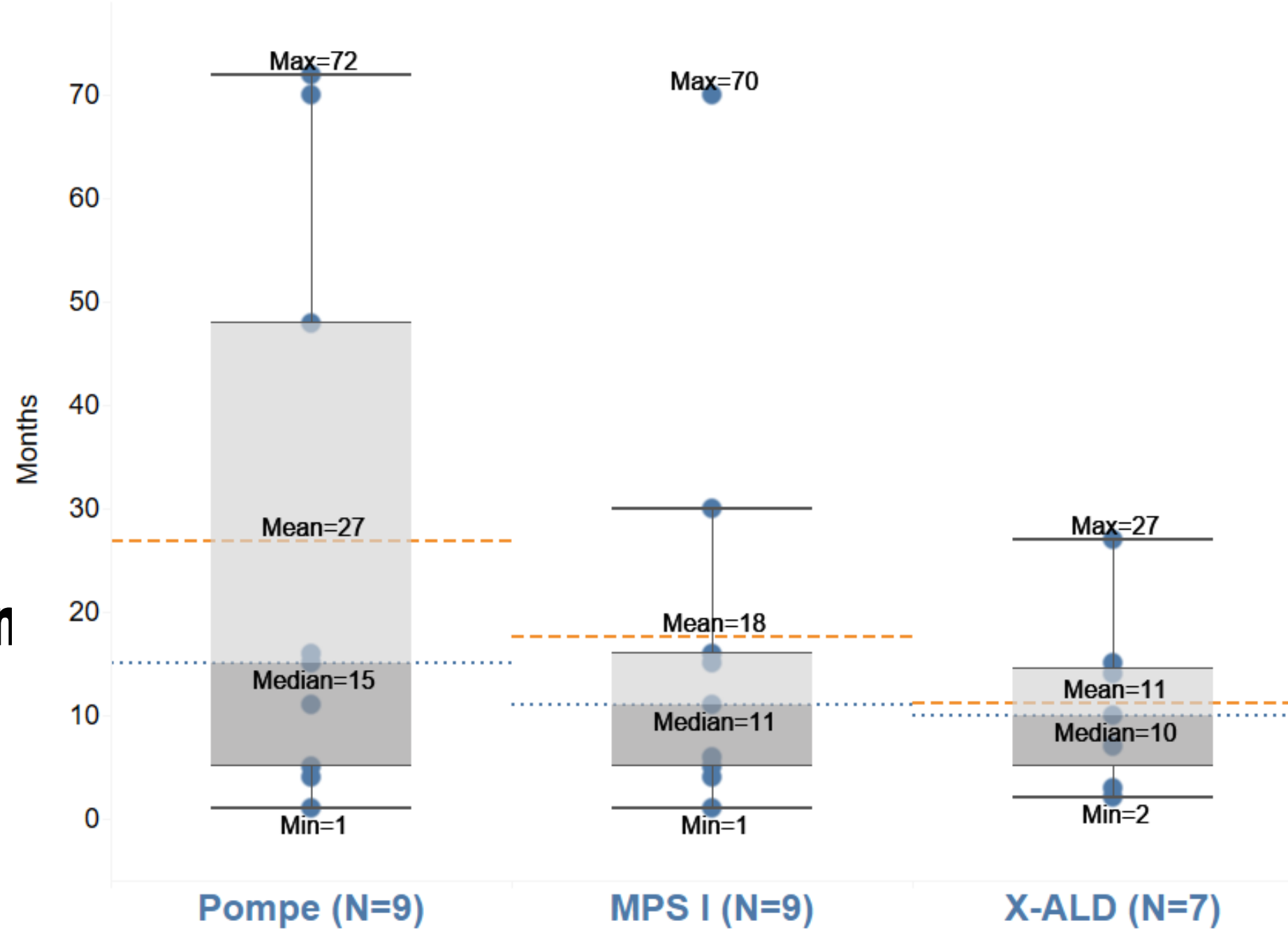
**42%-55%** of the participating programs with statewide screening for a new disorder were directed by their state legislation

# Burden on Families

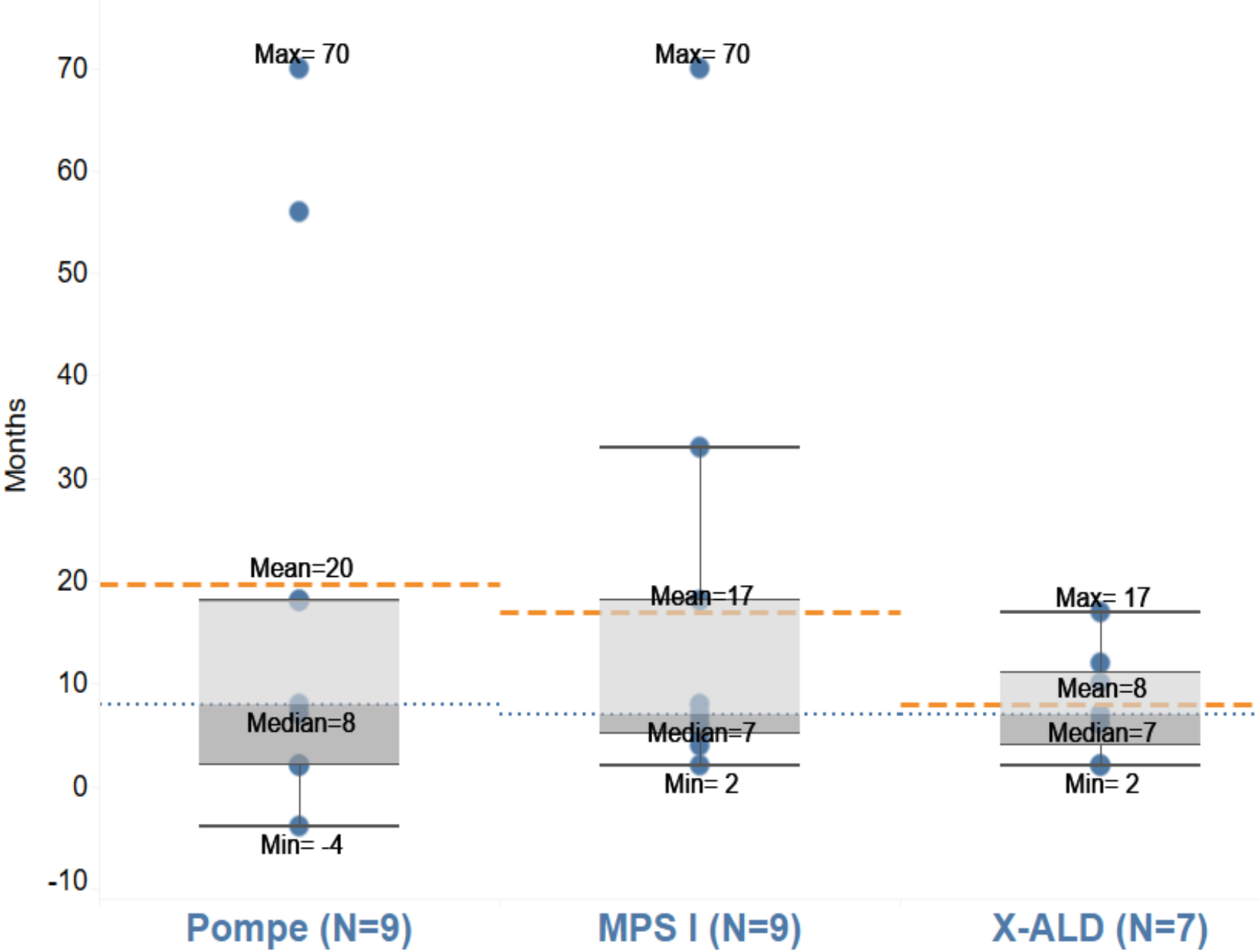
*“[It is] easy and cheap to add [new disorder], but puts follow-up in tough situation sometimes; specialists who don’t know what happens yet, finding pseudo-deficiencies . . . What if [follow-up] is not ready to bring a child through the process [of having a positive newborn screen], especially if false positive?”*

-Key Informant Interview

# Actual Time from Approval to Screen to Statewide Implementation



# Actual Time from Approval of Funding to Statewide Implementation



NOTE: Negative value for Pompe minimum is a result of approval for funding occurring after full implementation (i.e. 4 months after screening)