

Understanding Statewide Implementation of New Disorders

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Newborns and Children

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Overview

New Disorders Recently Added to the RUSP

New Disorder	Date added
Pompe	March 2, 2015
MPS I	February 16, 2016
X-ALD	February 16, 2016
SMA	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

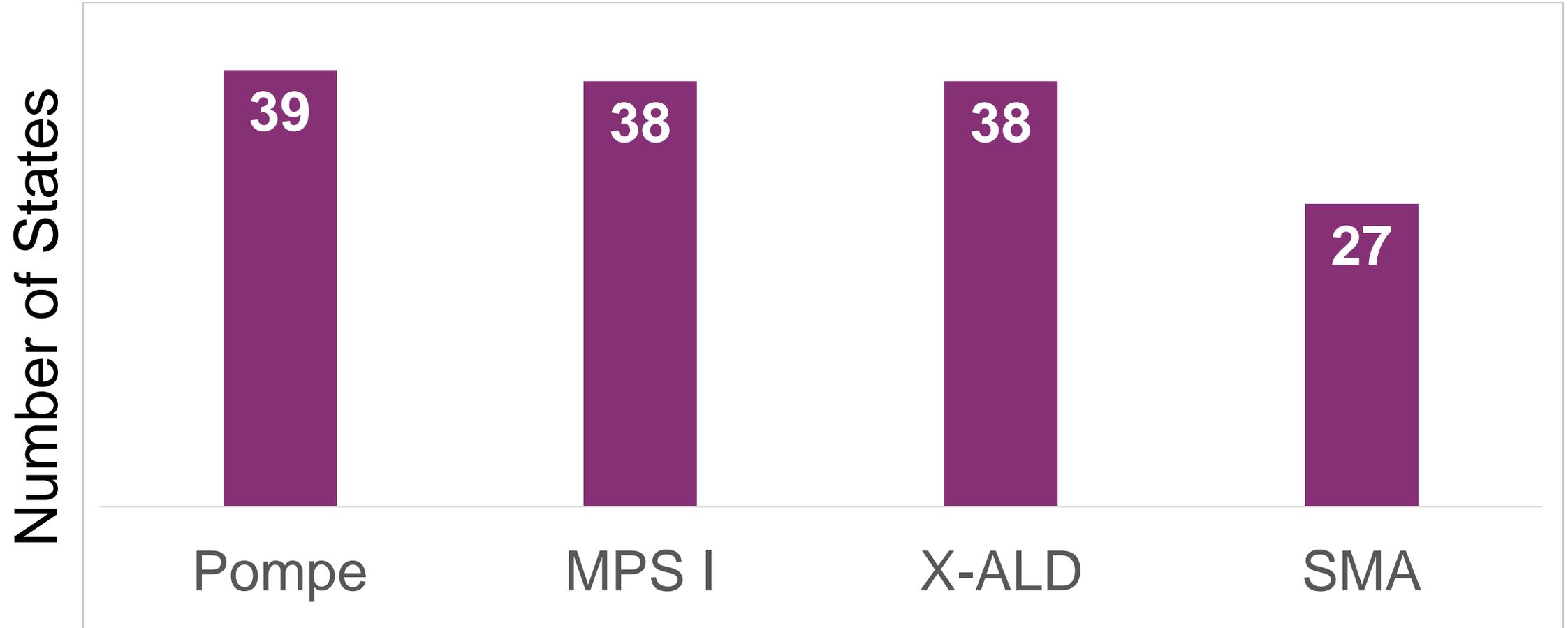
1. How long does it take to implement statewide screening for a new disorder?
 - a. How long does each readiness phase take?
 - b. Where is the most time spent?
2. What are the facilitators and challenges for statewide screening?

Background & Methods

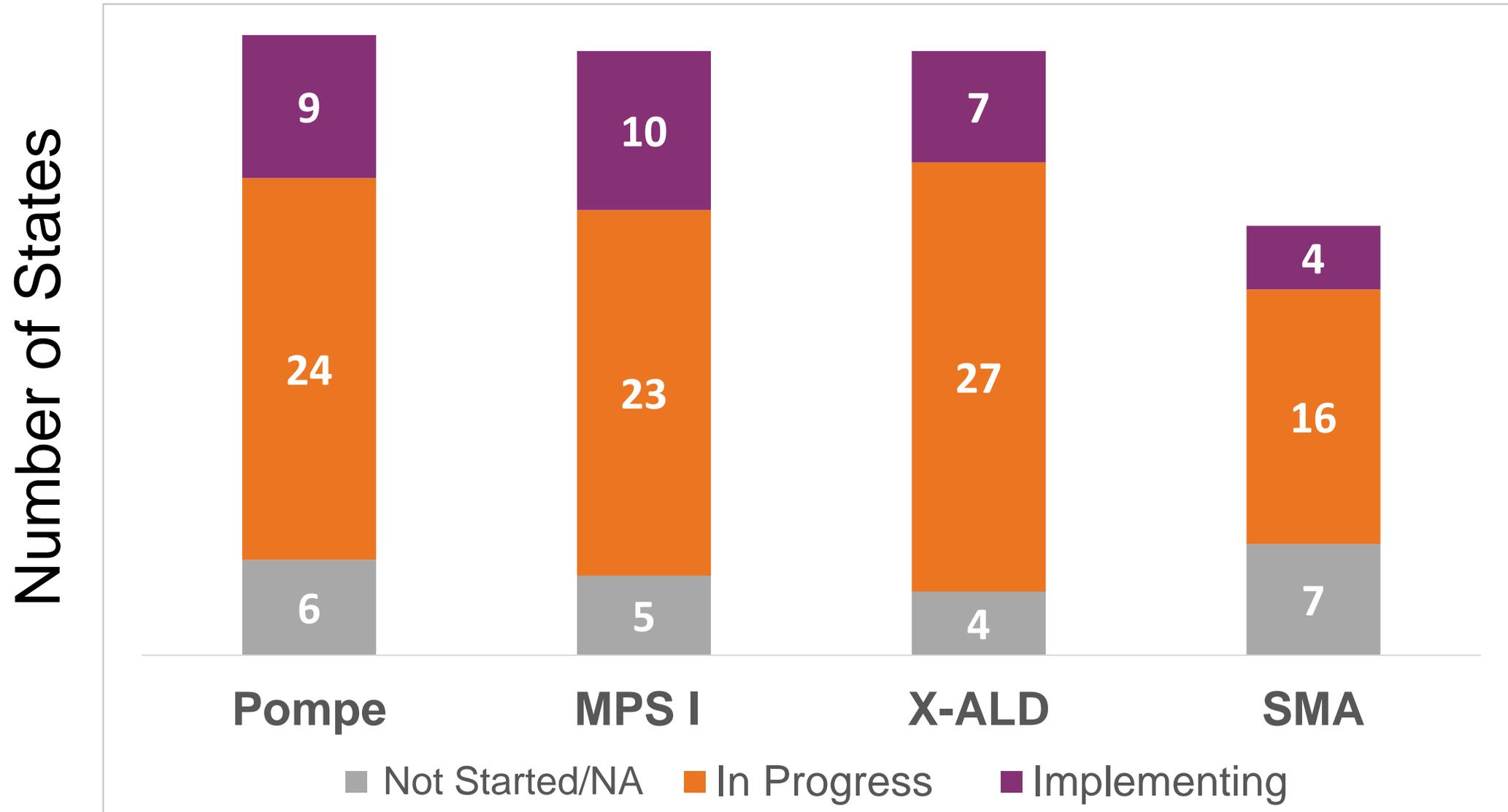
Methods to Assess Time

- **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>
Pompe	39	9 (23.1%)	21 (53.8%)	6 (15.0%)
MPS I	38	10 (26.3%)	19 (50.0%)	5 (13.2%)
X-ALD	38	7 (18.4%)	22 (57.9%)	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

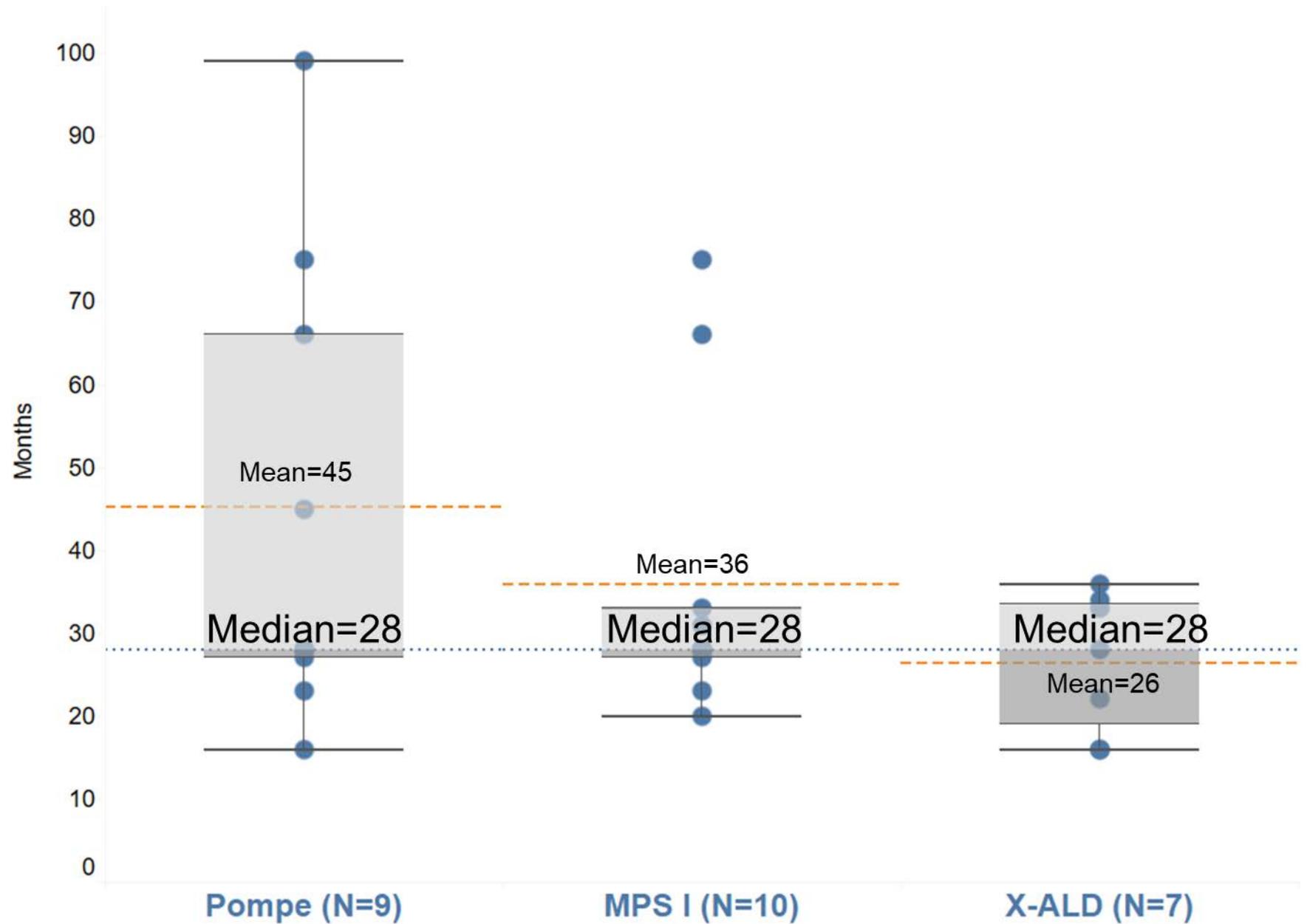
Limitations

- 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

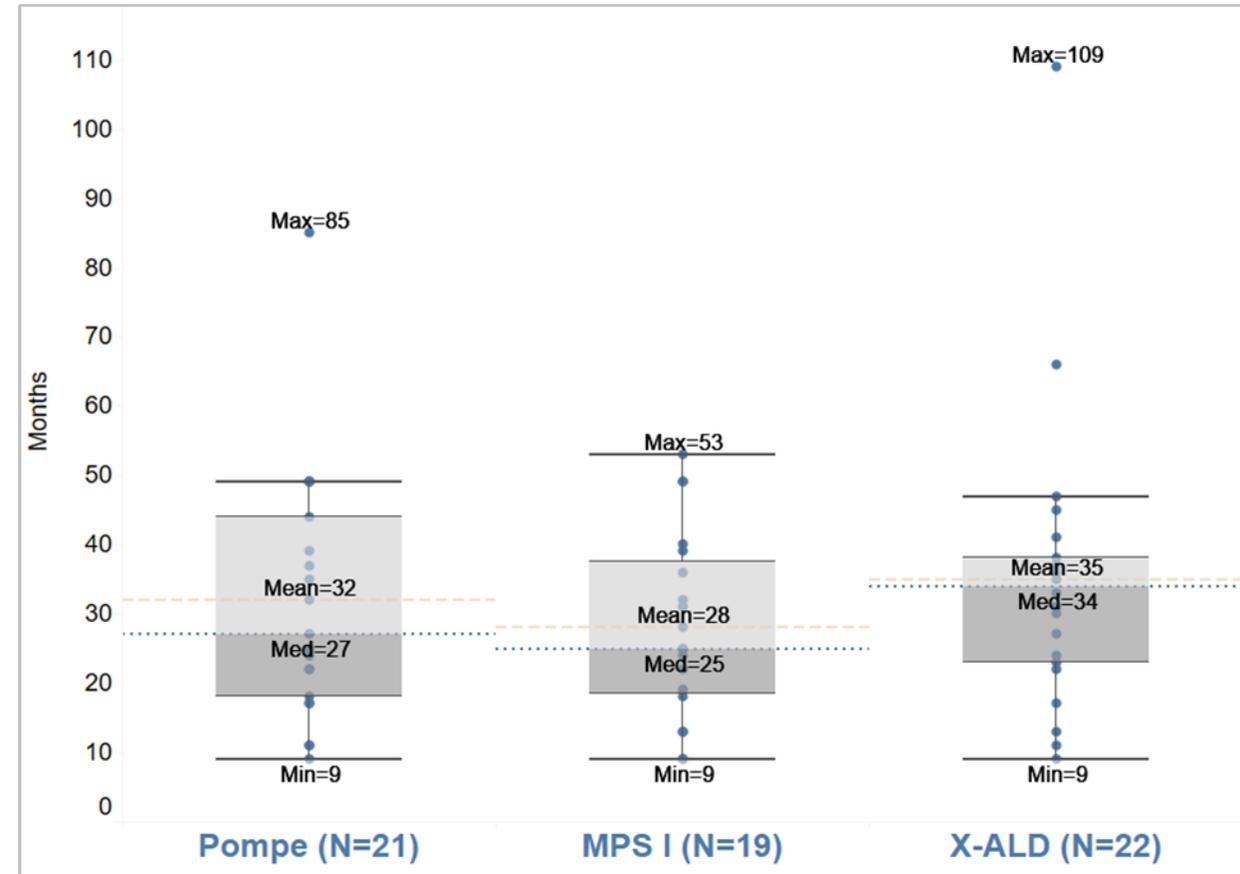
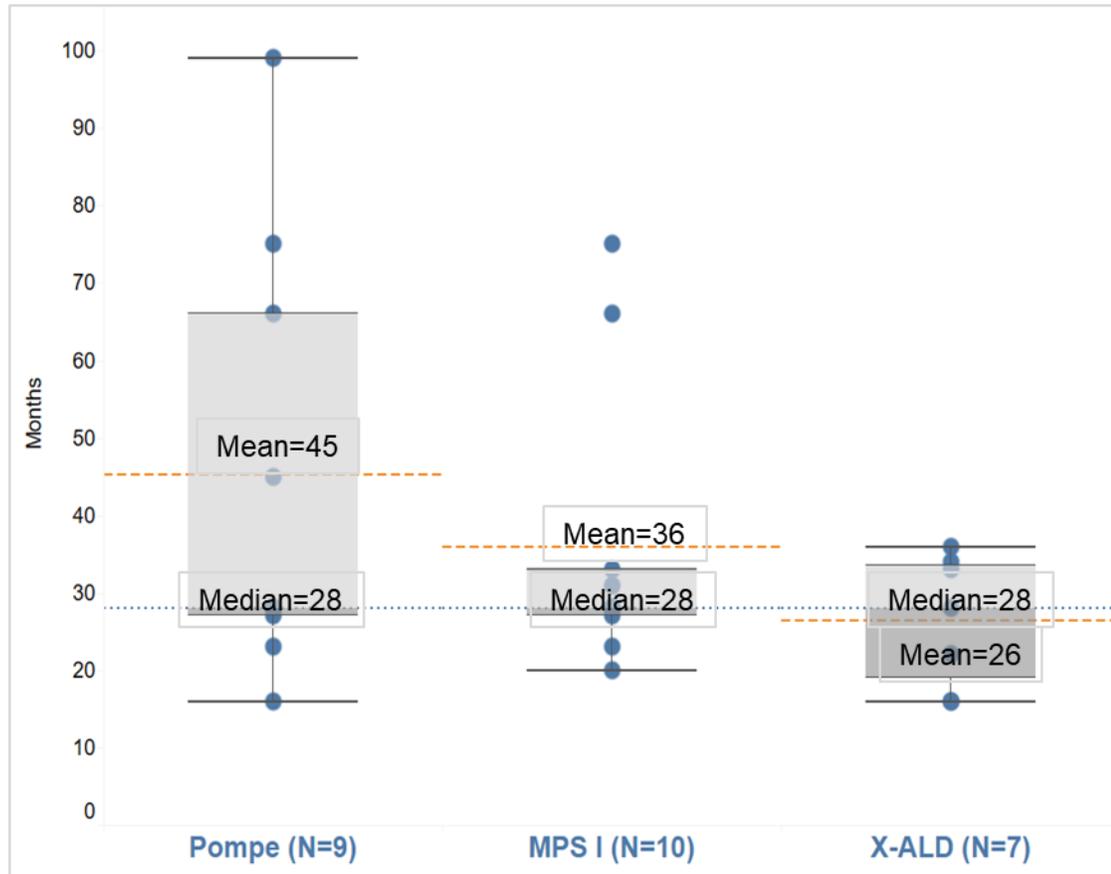


**How does
implementation time
compare for states that
are screening vs. those
that are in progress?**

Time to Statewide Screening (Implemented)

vs.

Time Spent working Towards Screening (In Progress)



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



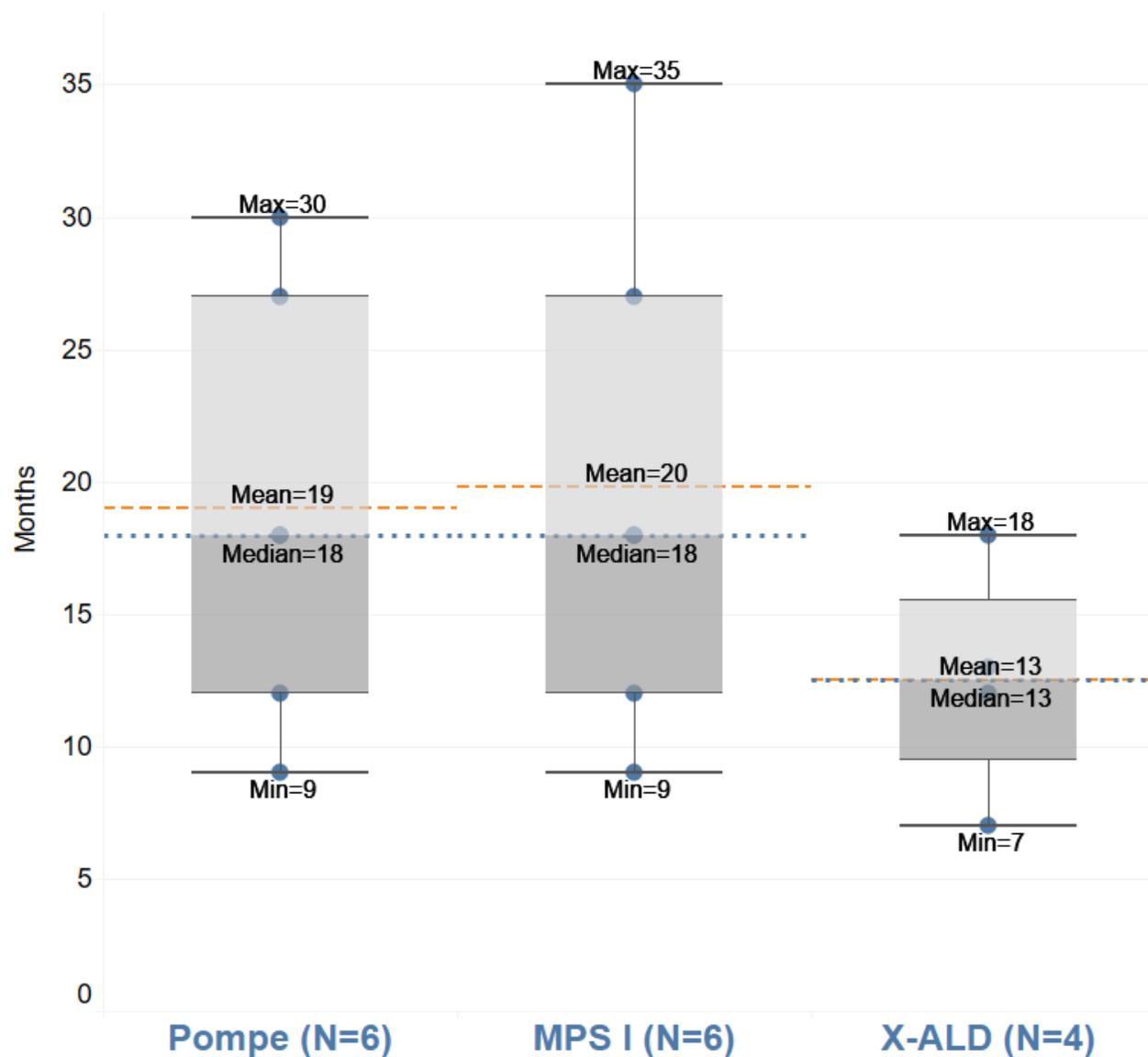
Phase 1 – Authority to Screen

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

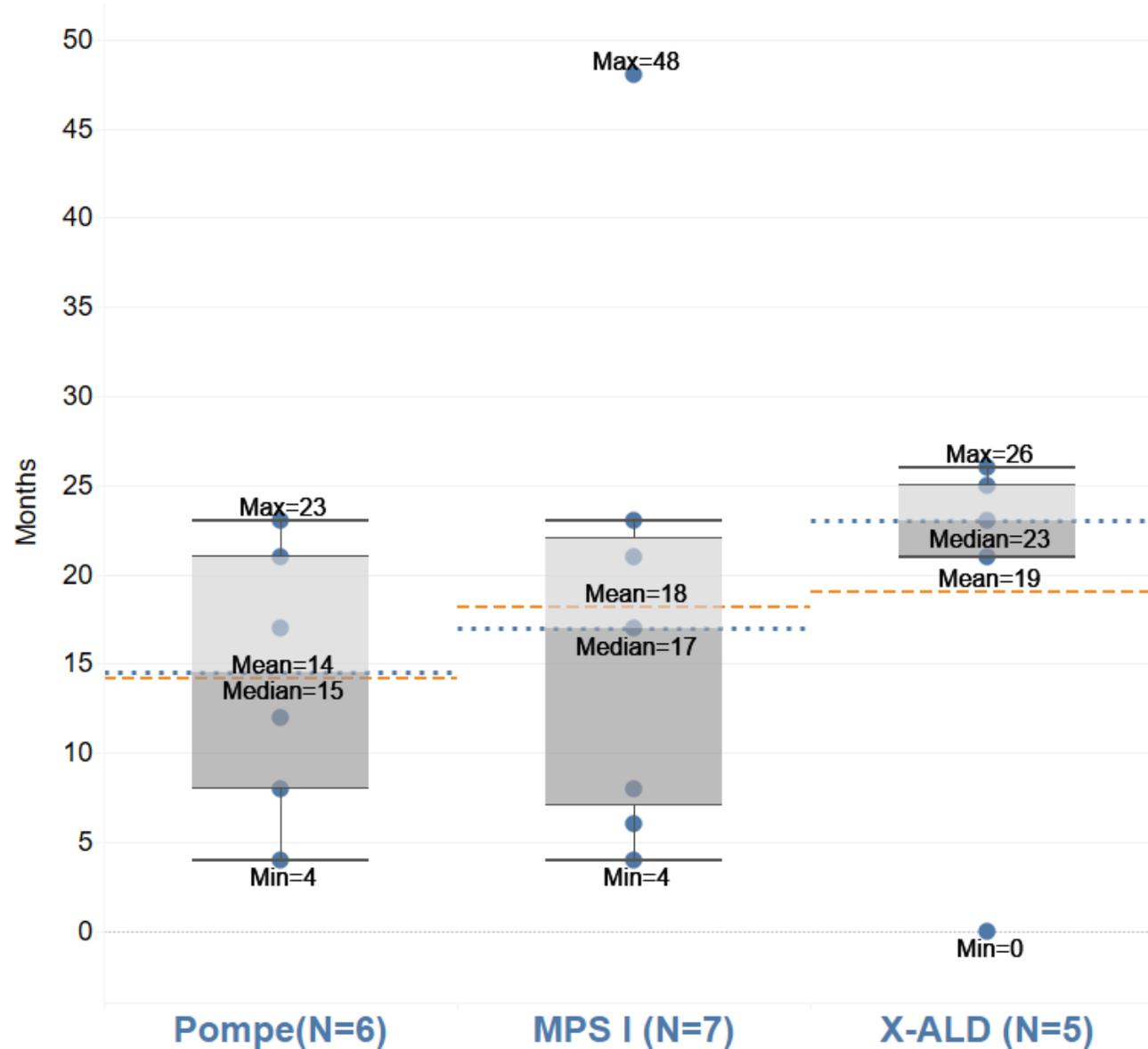
Phase 1a—Approval/Authority to Screen

For Programs that Implemented Statewide Screening



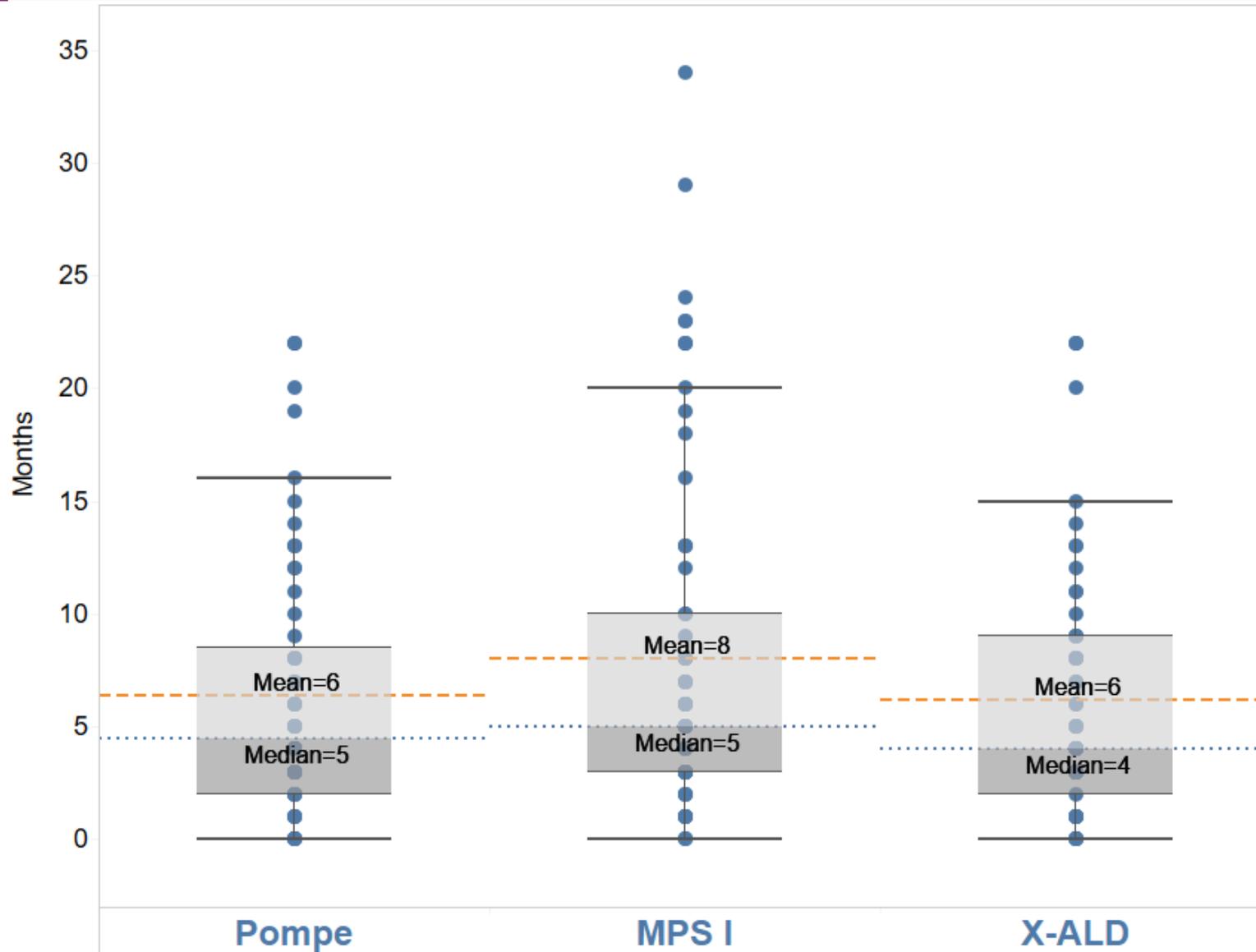
Phase 1b—Approval for Funding

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)

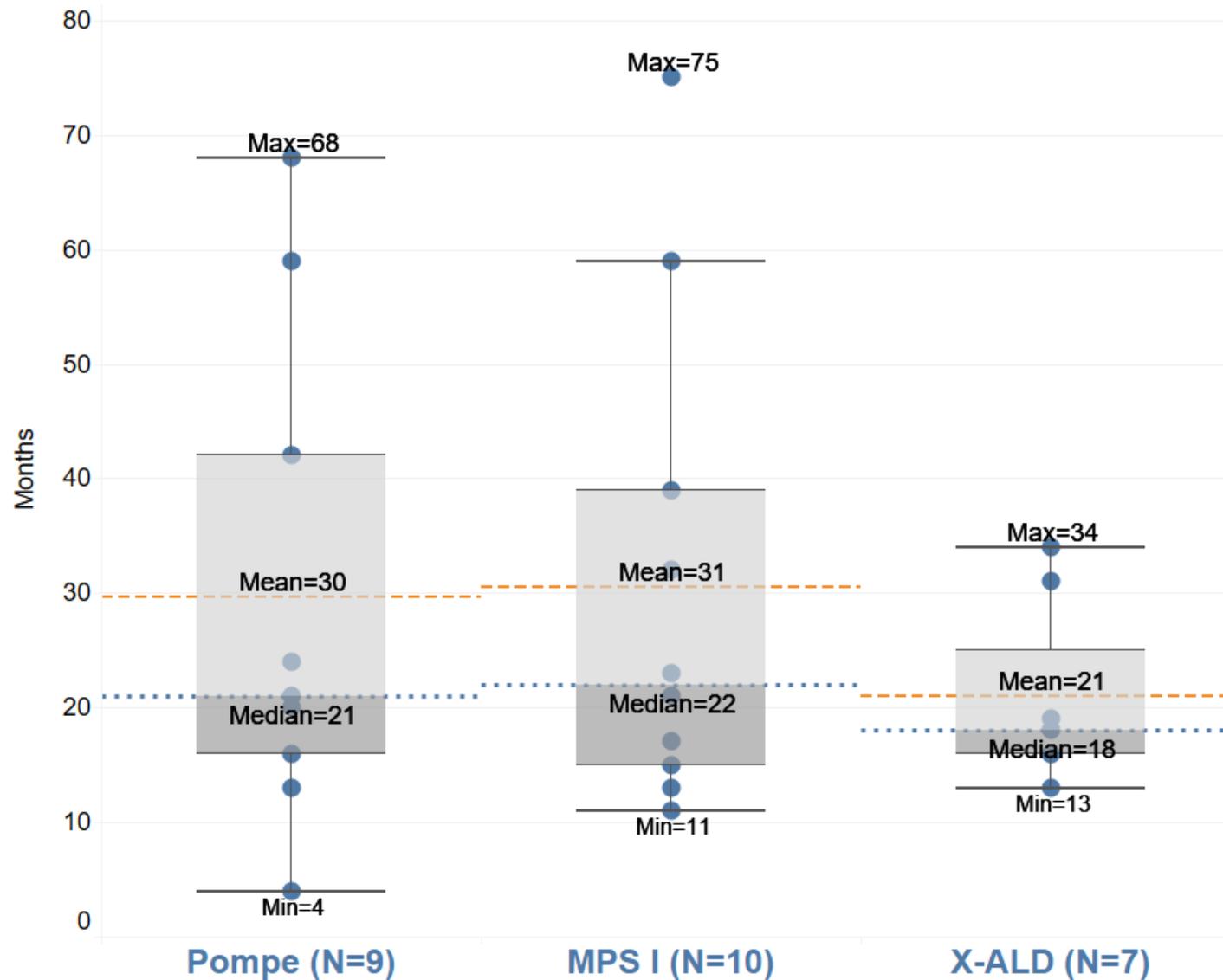
*“In [our] administrative code we review all new disorders that come onto the RUSP and report back to the full-advisory committee. That [state-based] committee will vote on recommendation . . . and send to the Commissioner of Health. [The] Commissioner will then take to the Board of Health and say [we] 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

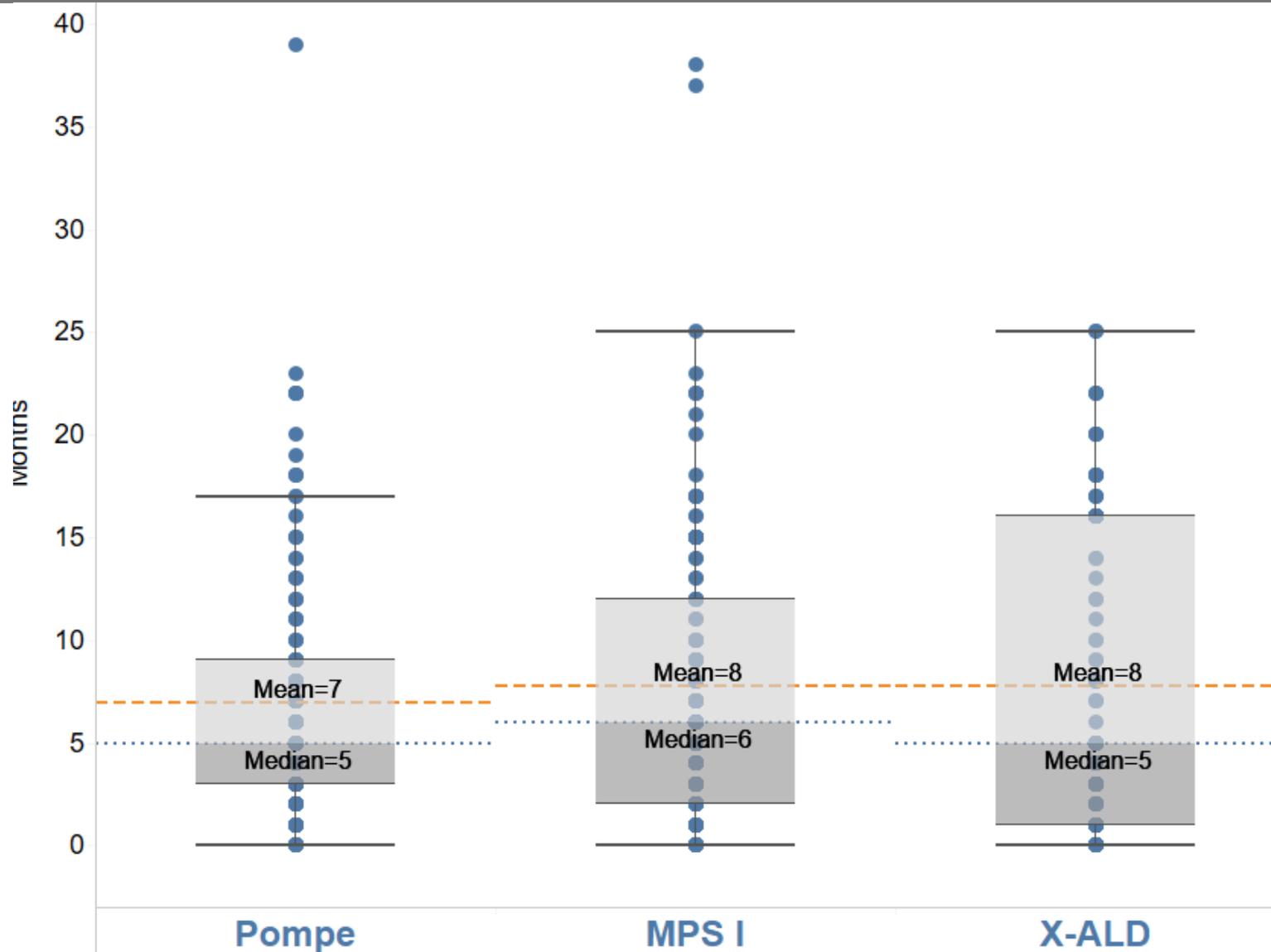
For Programs that Implemented Statewide Screening



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



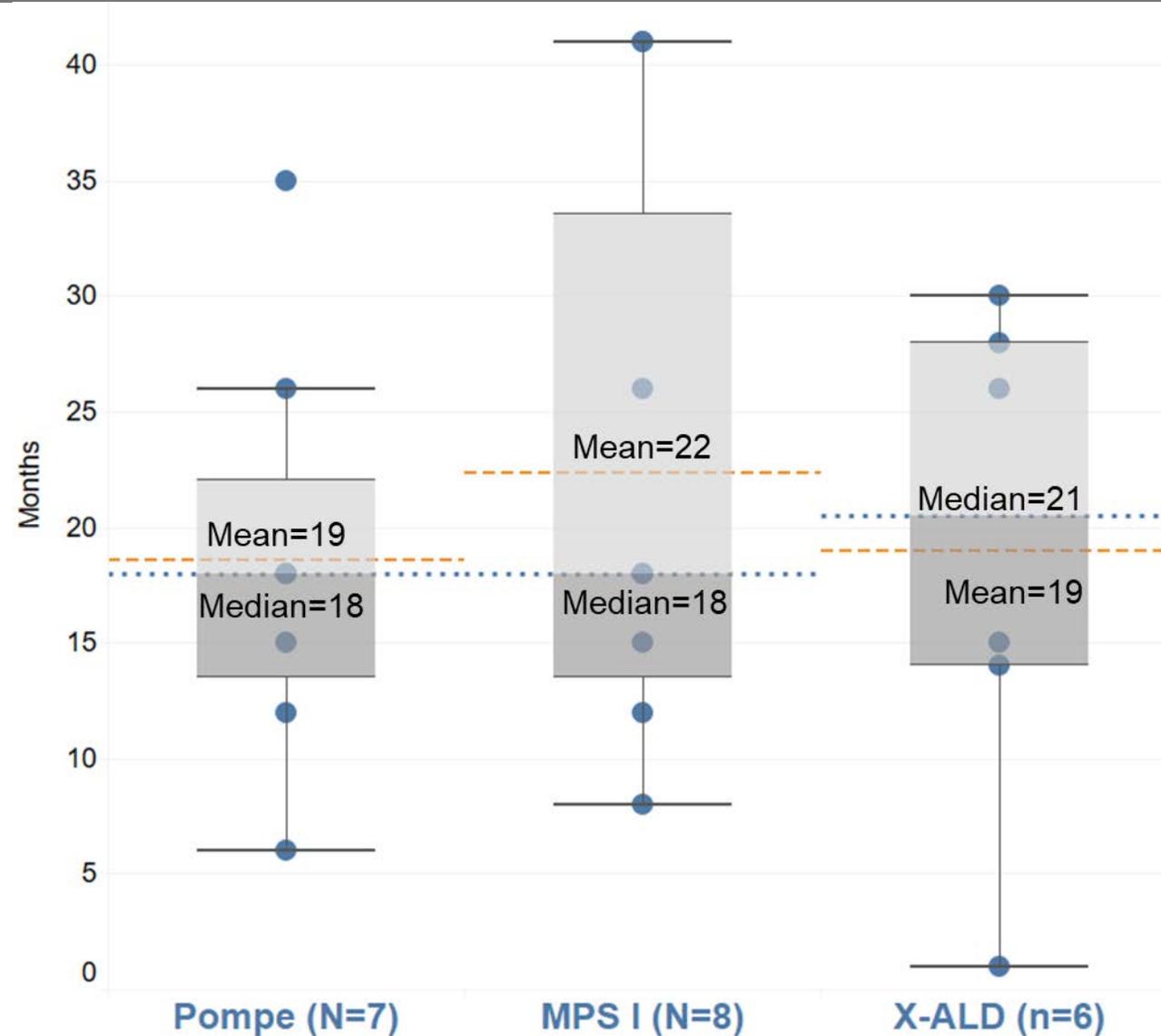
Validation of methodology was identified as a facilitator by 4 states



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Phase 2 – Follow-Up Readiness

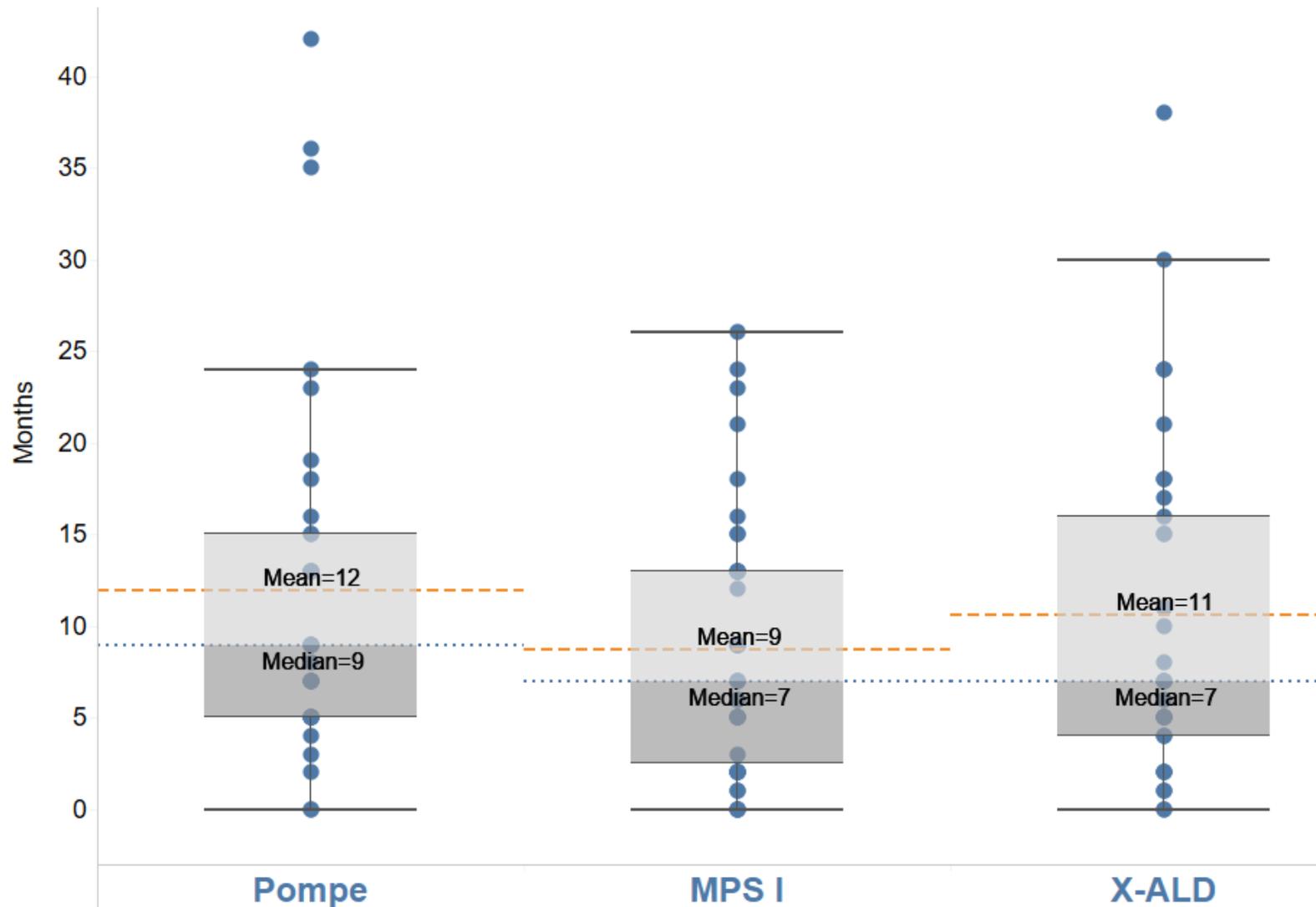
For Programs that Implemented Statewide Screening



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

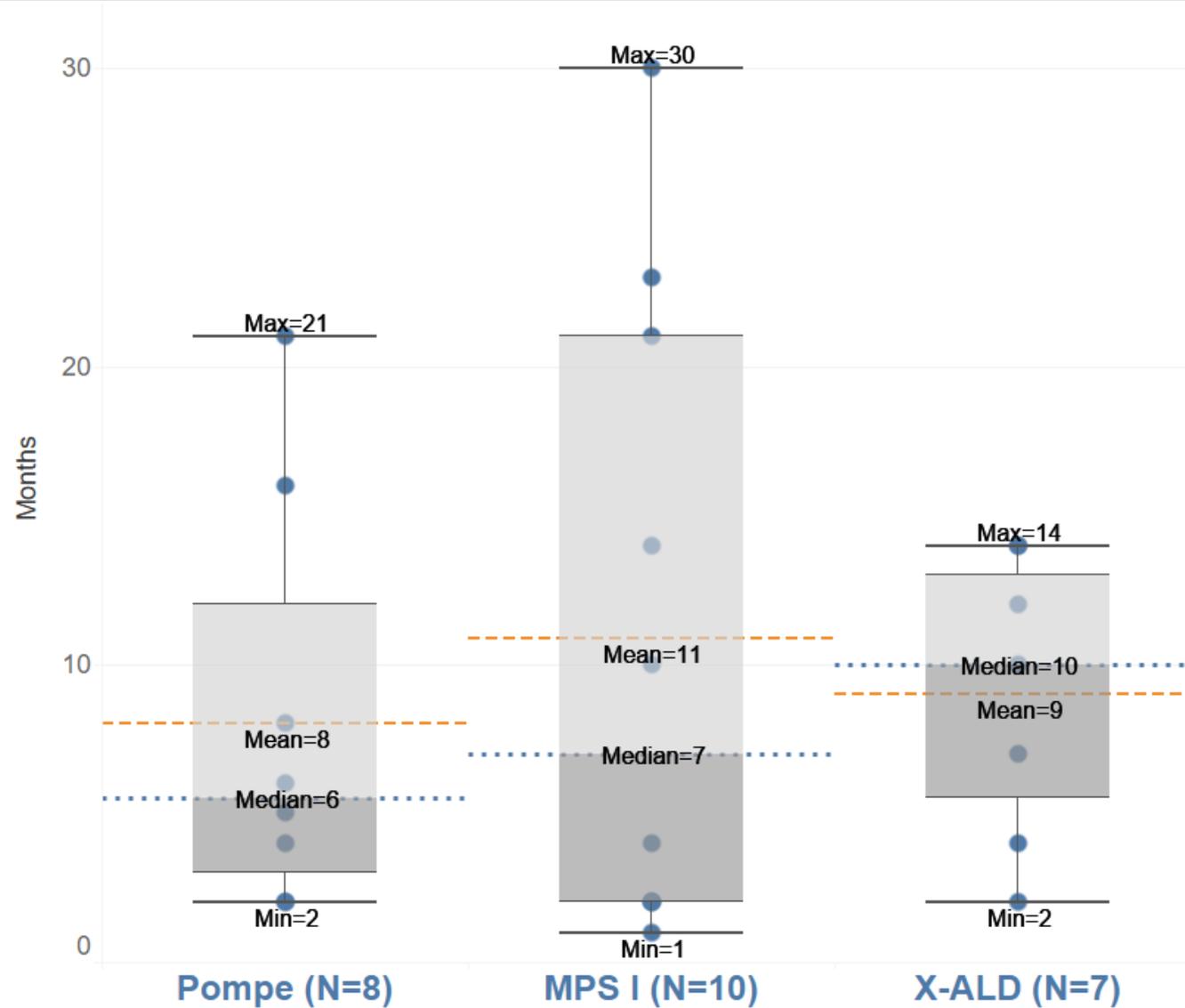


Phase 2 – IT Readiness

IT was the most frequently listed activity that started after implementation

Phase 2 – IT Readiness

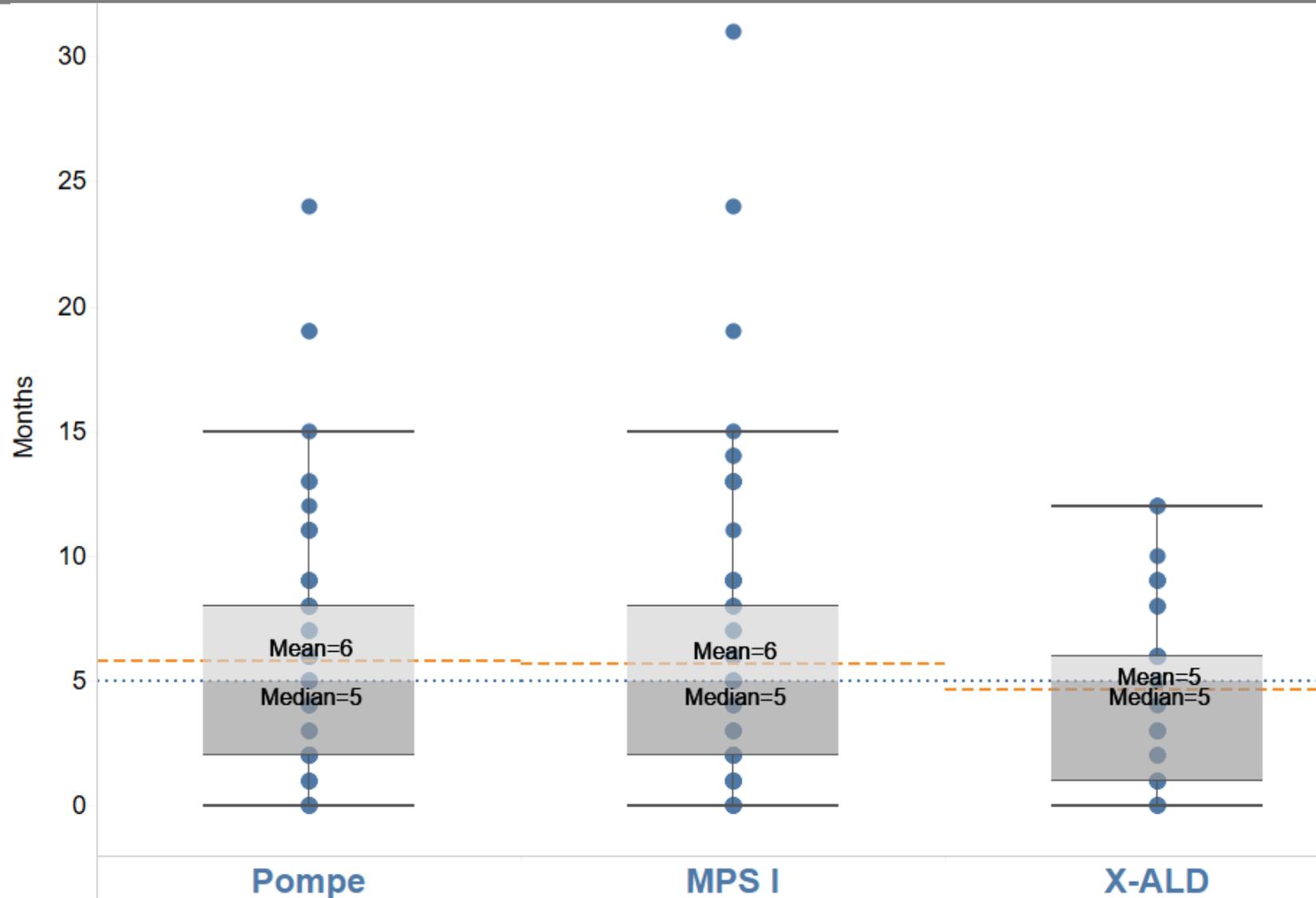
For Programs that Implemented Statewide Screening



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

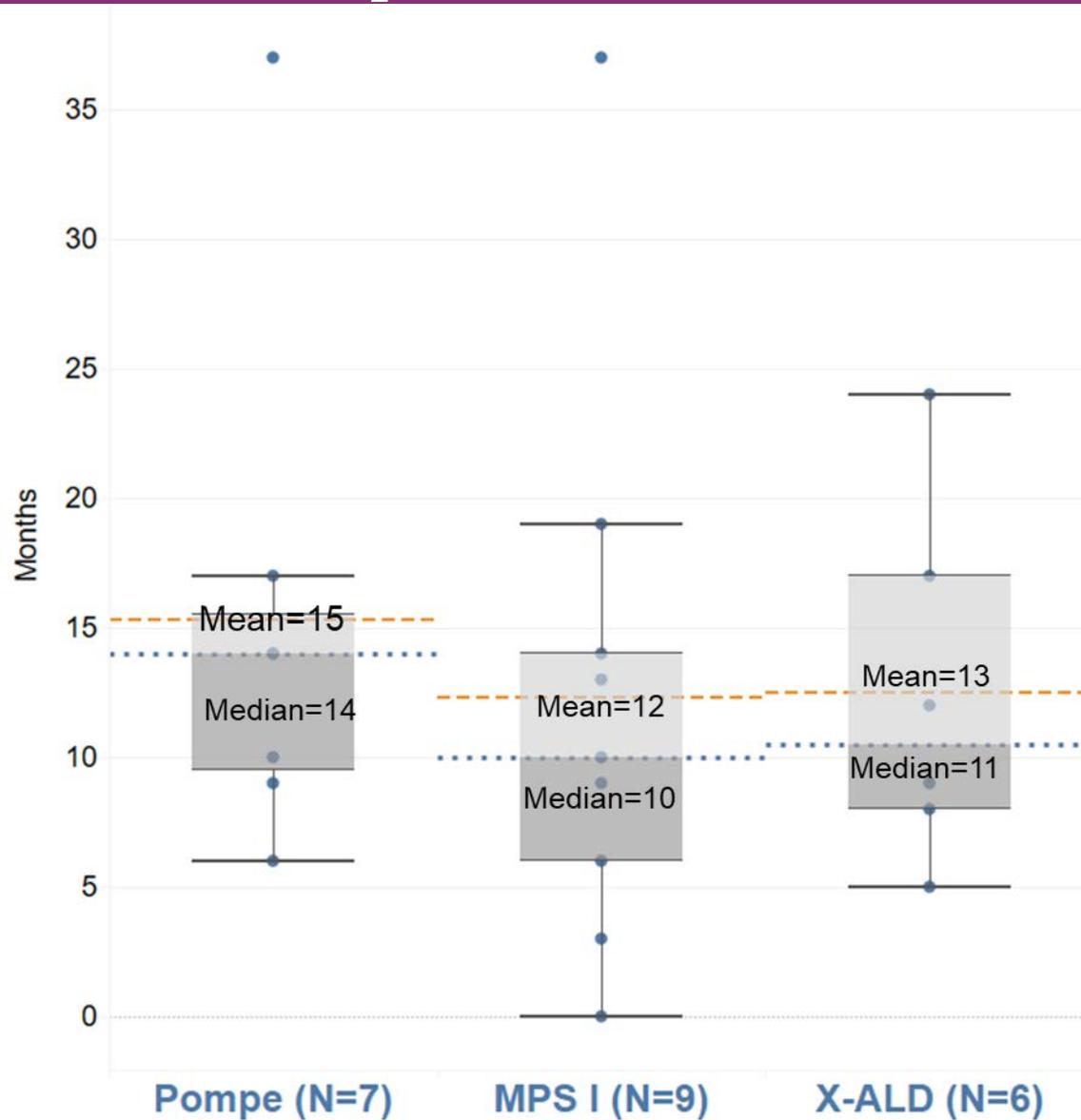
Data from Readiness Tool (N=20)



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Phase 3 – Education Readiness

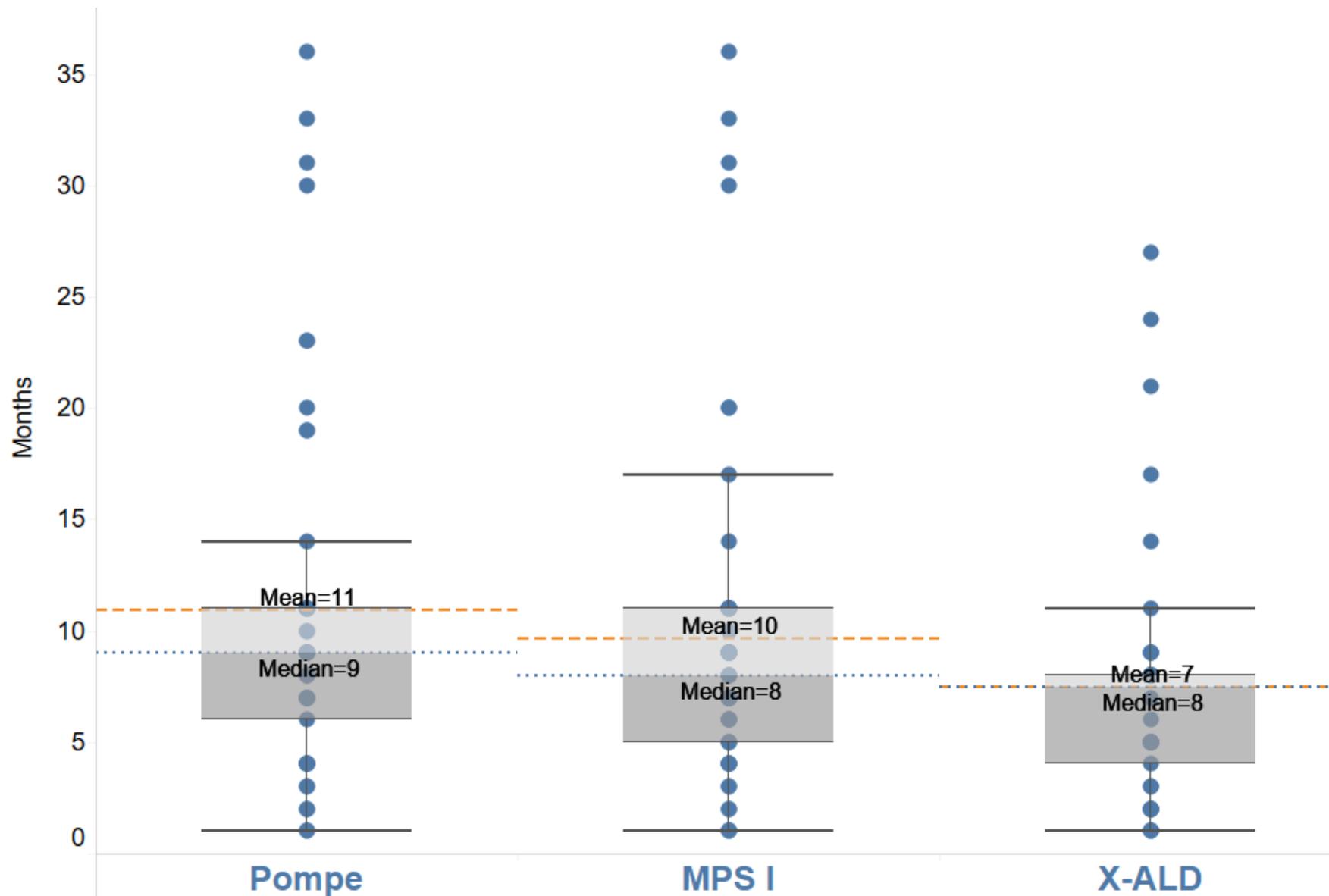
For Programs that Implemented Statewide Screening



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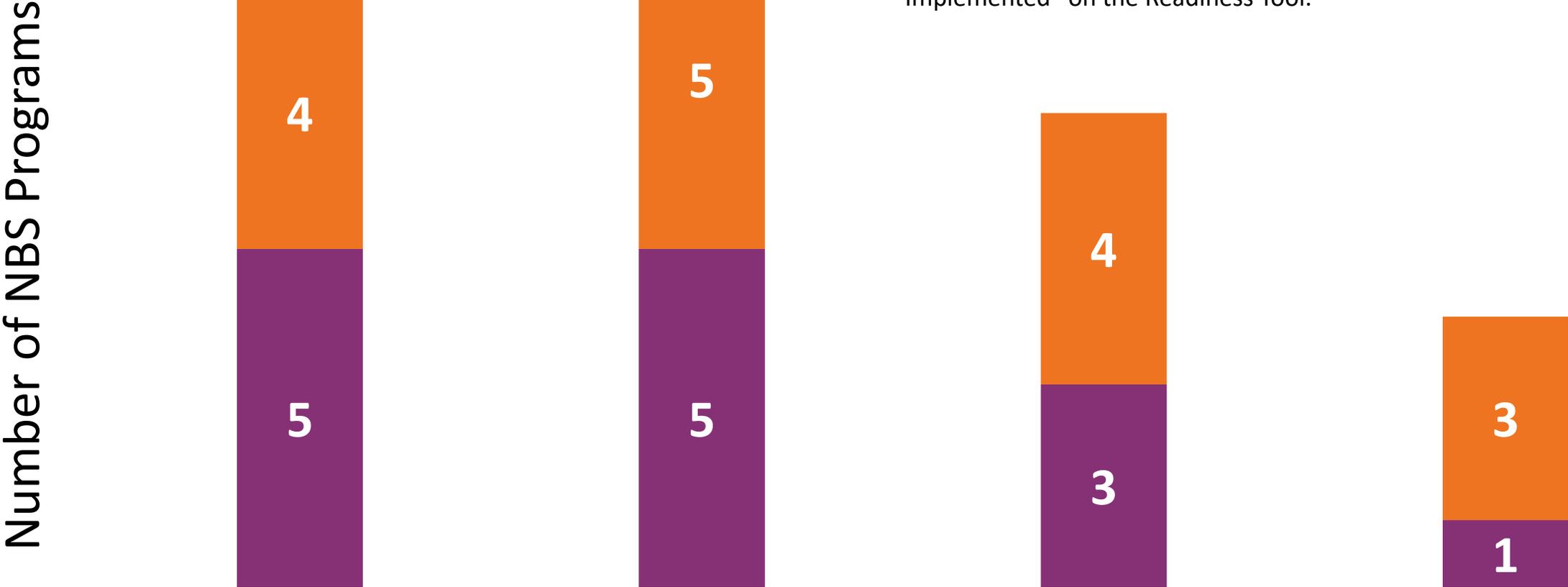
**Not Covered In Presentation But May Help
with Questions**

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.



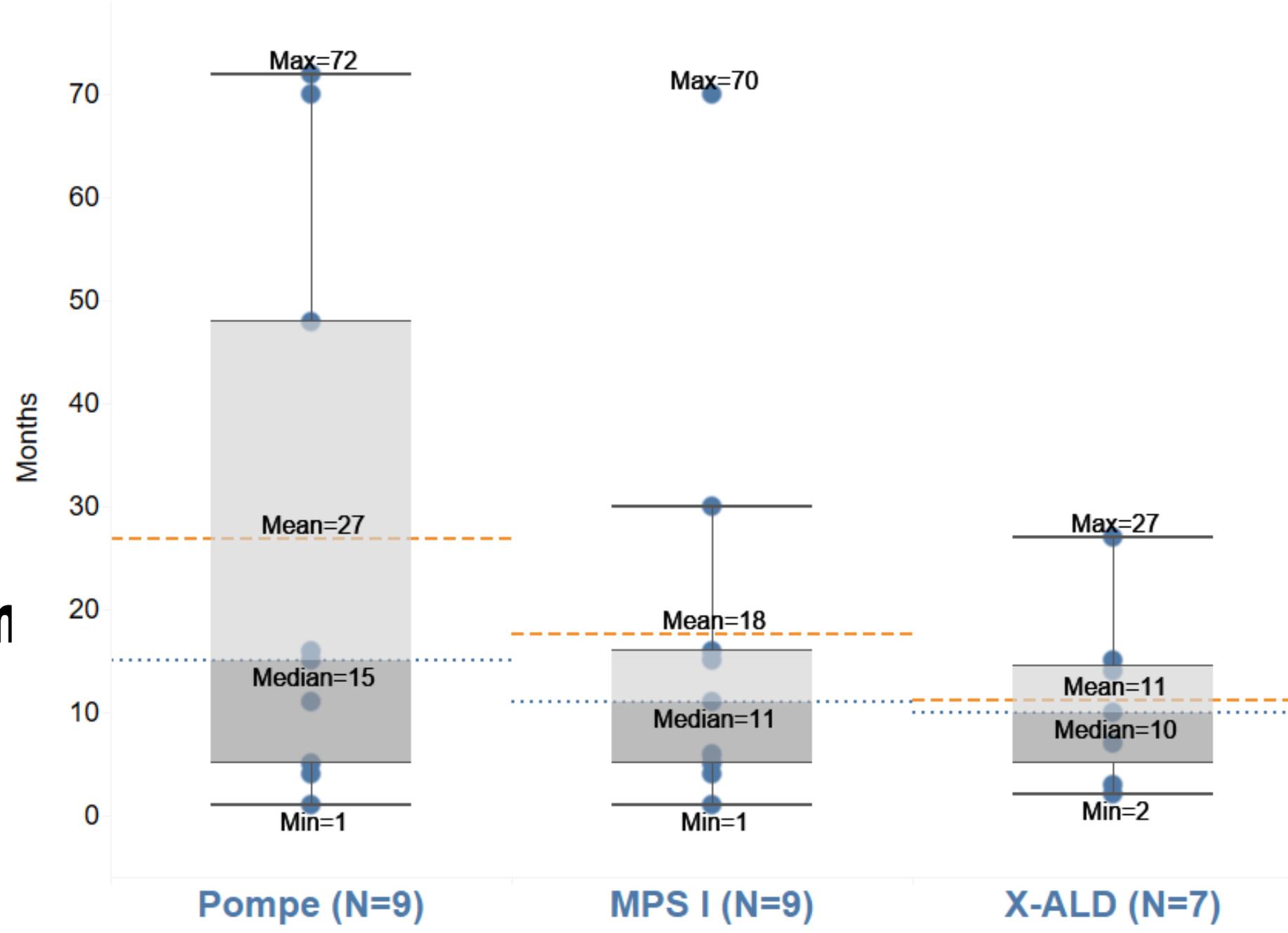
42%-55% of the participating programs with statewide screening for a new disorder were directed by their state legislation

Burden on Families

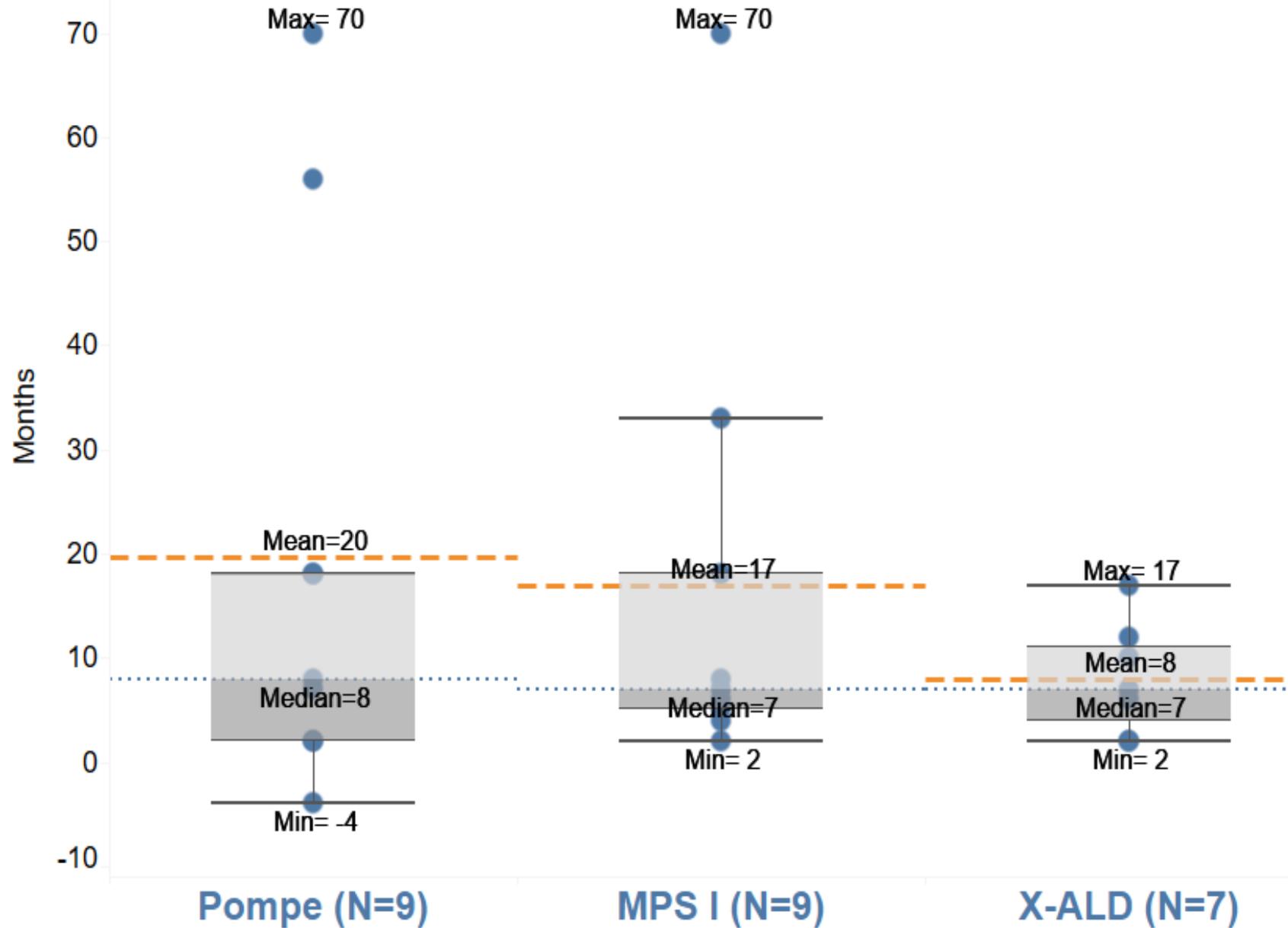
“[It is] easy and cheap to add [new disorder], but puts follow-up in tough situation sometimes; specialists who don’t know what happens yet, finding pseudo-deficiencies . . . What if [follow-up] is not ready to bring a child through the process [of having a positive newborn screen], especially if false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