State Wins to National Strategy

Advancing Newborn Screening Together

September 30, 2025

Supported by BioMarin
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RARE READY
A GENETIC CONDITION COALITION

This material was presented on 9/30/25; its continued accuracy, reliability, correctness, or completeness cannot not be assured after that date.

What We'll Cover Today

- Florida Sunshine Genetics Act
- The Future of Newborn Screening
- Newborn Screening Wins & Losses
- Looking Ahead

RARE READY
A GENETIC CONDITION COALITION

NEWBORN SCREENING
Florida Sunshine Genetics Act

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FSU | FLORIDA INSTITUTE FOR PEDIATRIC RARE DISEASES
COLLEGE OF MEDICINE

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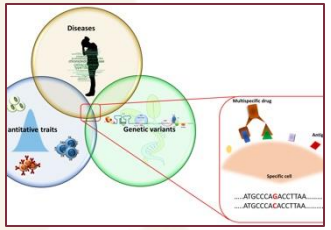
FSU | FLORIDA INSTITUTE FOR PEDIATRIC RARE DISEASES

Our mission is to advance the diagnosis, treatment, and prevention of pediatric rare diseases through cutting-edge research, precision medicine, and clinical care, and position Florida to be the national leader in precision medicine

1

Research Programs



Rare Disease Research Program

2

Training Programs

Graduate Program in Genetic Counseling

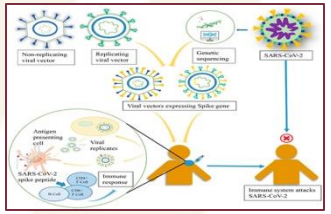
3

Clinical Diagnostics

CLIA-CAP Genomics Laboratory Facility

4

Genetic Engineering



Viral Vector and Gene Editing Facility

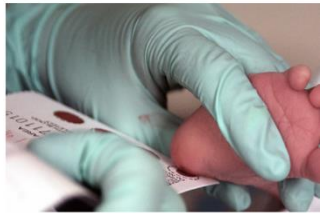
5

Precision Medicine

IPRD Pediatric Clinical Service

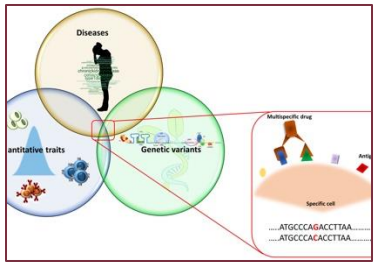
6

Sunshine Genetics



Newborn Whole Genome Sequencing

Rare Disease Research Grants Program



Program Goals

- Identify and support rare disease research groups across FSU campus
- Encourage collaboration
- Support pilot projects to advance research toward extramural funding
- Publications, Conference Presentations, IP

Master's Program in Genetic Counseling

STATE UNIVERSITY SYSTEM OF FLORIDA

Request to Offer a New Degree Program

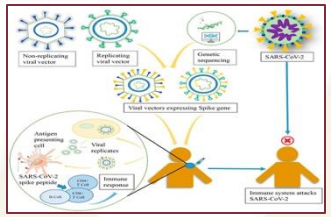
In accordance with Board of Governors Regulation 8.011, Academic Degree Program Coordination and Approval

Florida State University Institution Submitting Proposal	Fall 2026 Proposed Implementation Term
College of Medicine Name of College(s) or School(s)	N/A Name of Department(s)/Division(s)
Genetic Counseling Academic Specialty or Field	Genetic Counseling MS Complete Name of Degree
51.1509 Proposed CIP Code (2020 CIP)	

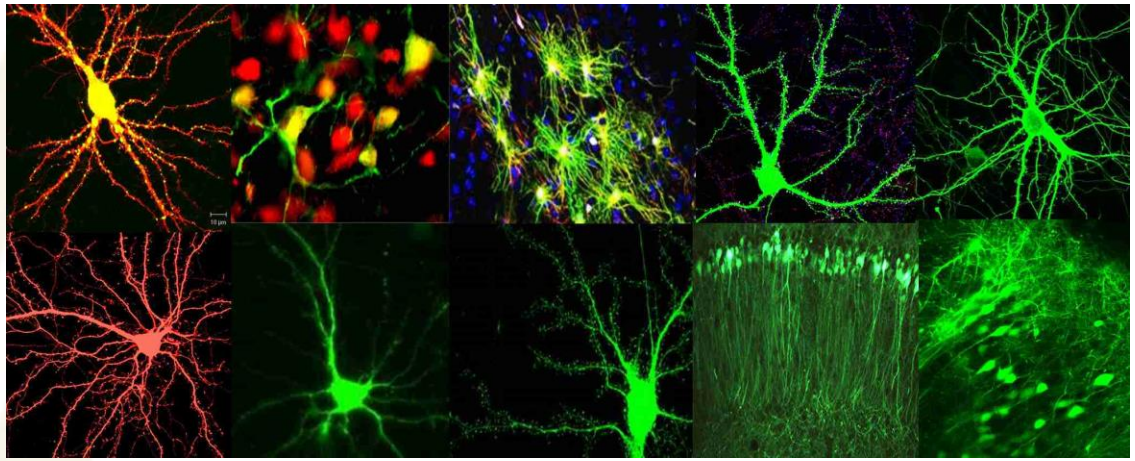
The submission of this proposal constitutes a commitment by the university that, if the proposal is approved, the necessary financial resources and the criteria for establishing new programs have been met before the program's initiation.

9/13/24 Date Approved by the University Board of Trustees	9/13/24 President's Signature — Date
9/13/24 Board of Trustees Chair's Signature — Date	9/13/24 Provost's Signature — Date

1. Program approved by the University Board of Trustees on September 13, 2024
2. Efforts are underway to recruit a Program Director
3. Recruited a genetic counselor from UCLA (started August 14, 2025)
4. Rep. Anderson is planning legislation to provide **educational loan forgiveness** and **healthcare provider status** under Medicaid and commercial insurers (i.e., reimbursement for services) to **genetic counselors**



Viral Vector and Gene Editing Facility



A full-service facility specializing in the **design, development, and production** of viral vectors and **gene editing** tools. We support gene editing and gene therapy needs of **academic, clinical, and industry partners** across the nation.

CLIA-CAP Certified Genomics Diagnostics Facility

A Strategic Partnership with Quest Diagnostics

State-of-art genomics ***clinical diagnostic service***
at competitive rates and industry leading
turnaround time

Pediatrics Clinical Service

- Multidisciplinary clinical care to children with undiagnosed diseases, complex medical problems, autism or other neurodevelopmental disorders (developmental delay, intellectual disability, epilepsy, cerebral palsy, motor speech disorders)
- A “genome-first” approach using diagnostic whole genome sequencing on first visit to shorten the “diagnostic odyssey” and initiate early intervention and triage to appropriate pediatric specialists
- Serve as a hub for patient recruitment into research studies

Sunshine Genetics Newborn WGS Pilot Program

- The **Sunshine Genetics Act** supports newborn **whole genome sequencing**
- Families can volunteer (full opt-in consent) to have their baby's genome sequenced and screened for ~250-600 conditions in addition to traditional NBS ~60 conditions.
- The goal is to identify potentially serious but treatable conditions early, so that care can begin before symptoms appear

Sunshine Genetics Newborn WGS Pilot Program

- A **Board of Overseers** representing Florida's Universities, Health Care Institutions and the Government.
- A **Sunshine Genetics Consortium** to execute the pilot program and promote collaboration among hospitals, universities, industry and genetic researchers. A **Steering Committee** of the consortium will be established to determine:
  - 1) Which genes/conditions to be screened for (range 250-600)
  - 2) When and how to recruit/consent patients (e.g., during pregnancy, at birth, pediatric well-baby visits)
  - 3) To develop standards for genome sequencing performance and quality metrics for central sequencing lab or multiple, distributed labs).

Sunshine Genetics Newborn WGS Pilot Program

- Develop a system for **secure data storage and sharing**, using only de-identified information for future research.
- Other considerations:
  - Close partnership with Florida DOH NBS Program and Lab to clearly educate patient-participants that this supplements traditional NBS and does not replace it.
  - Piggy-back in collaborative way to improve awareness of traditional NBS, leverage existing DBS collected for NBS, partner with NBS follow-up programs to expand capacity to return WGS positive results, provide genetic counseling and make appropriate medical referrals.

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COLLEGE OF MEDICINE

Thank you!

NEWBORN SCREENING

The Future

Stephen Kingsmore, M.D., DSc

President/CEO, Rady Children's Institute for Genomic Medicine

Rady Children's Health

How Whole Genome Sequencing (WGS) is Transforming the Field

Stephen Kingsmore MB, ChB, BAO, DSc, FRCPath

Rady Children's Institute Genomic Medicine®

1st Rapid Diagnostic WGS (rWGS)

Rady Children's Institute Genomic Medicine®

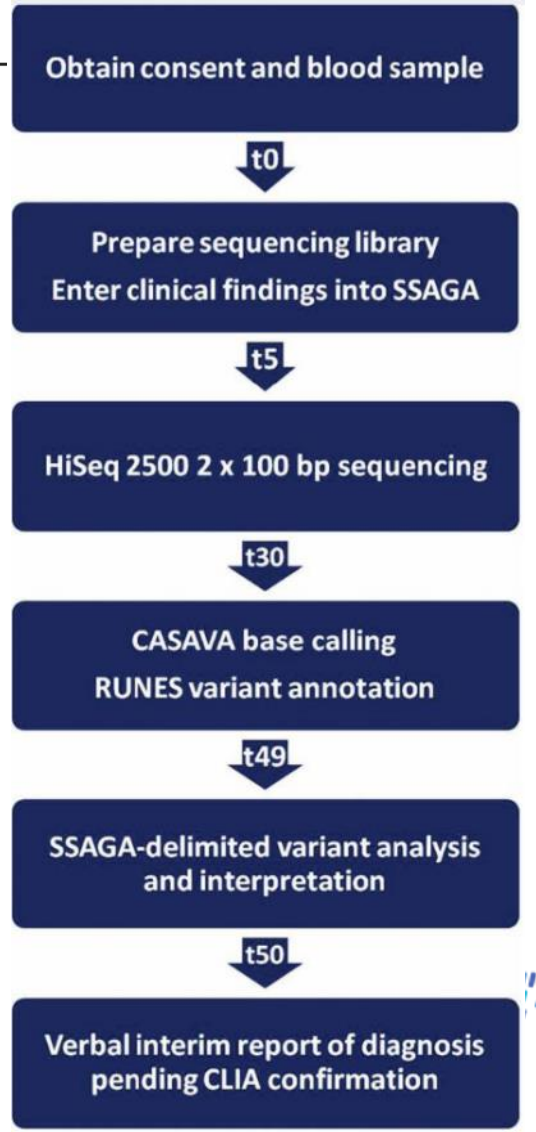
RESEARCH ARTICLE

Sci Transl Med **4**, 154ra135 (2012)

DIAGNOSTICS

Rapid Whole-Genome Sequencing for Genetic Disease Diagnosis in Neonatal Intensive Care Units

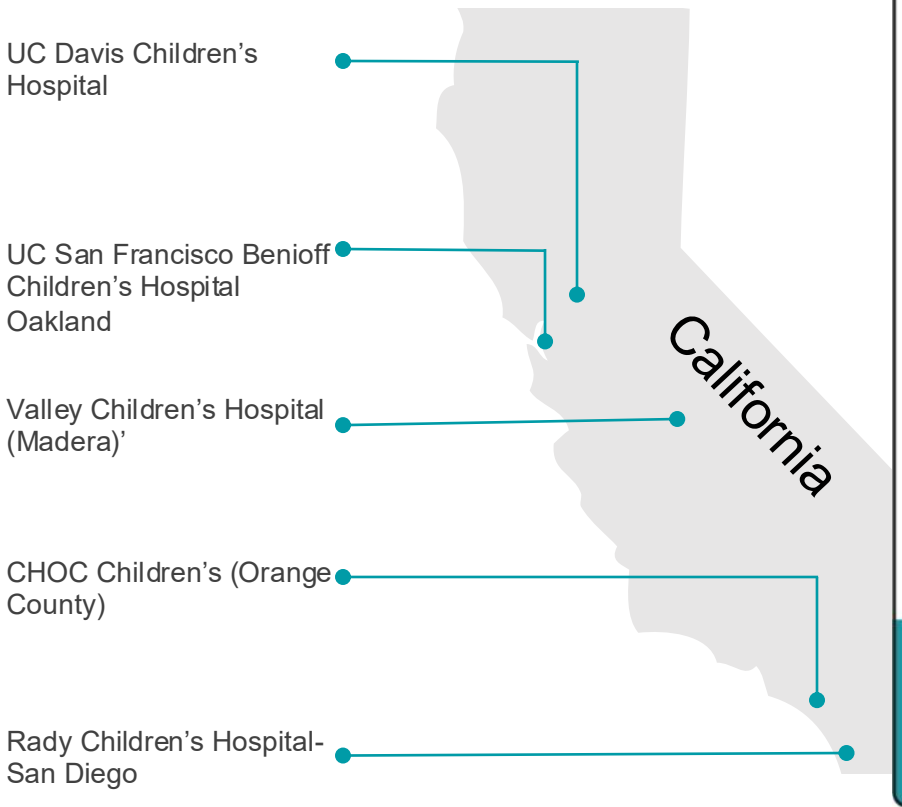
Carol Jean Saunders,[1,2,3,4,5]* Neil Andrew Miller,[1,2,4]* Sarah Elizabeth Soden,[1,2,4]* Darrell Lee Dinwiddie,[1,2,3,4,5]* Aaron Noll,[1] Noor Abu Alnadi,[4] Nevene Andraws,[3] Melanie LeAnn Patterson,[1,3] Lisa Ann Krivohlavek,[1,3] Joel Fellis,[6] Sean Humphray,[6] Peter Saffrey,[6] Zoya Kingsbury,[6] Jacqueline Claire Weir,[6] Jason Betley,[6] Russell James Grocock,[6] Elliott Harrison Margulies,[6] Emily Gwendolyn Farrow,[1] Michael Artman,[2,4] Nicole Pauline Safina,[1,4] Joshua Erin Petrikin,[2,3] Kevin Peter Hall,[6] Stephen Francis Kingsmore[1,2,3,4,5]†



- 7 years of development
- In 50 hours rWGS provided likely diagnoses in 4 of 5 critically ill newborns.

It took Implementation Pilot studies to achieve Medicaid coverage of rWGS in Neonatal Intensive Care Units

Rady Children's Institute Genomic Medicine®



PILOT SITES	# OF BABIES	BABIES DIAGNOSED	BABIES WHOSE CARE WAS CHANGED*	DAYS TO RESULTS**
CHOC CHILDREN'S HOSPITAL (ORANGE COUNTY)	23	12 (52%)	9 (39%)	2.5
RADY CHILDREN'S HOSPITAL-SAN DIEGO	59	22 (37%)	19 (32%)	3
UC DAVIS CHILDREN'S HOSPITAL *(Sacramento)*	34	12 (35%)	8 (24%)	2
UCSF BENIOFF CHILDREN'S HOSPITAL OAKLAND	24	12 (50%)	9 (38%)	3
VALLEY CHILDREN'S HOSPITAL *(Madera)*	38	18 (47%)	10 (26%)	3
TOTAL PROJECT BABY BEAR CASES	178	76 (43%)	55 (31%)	3

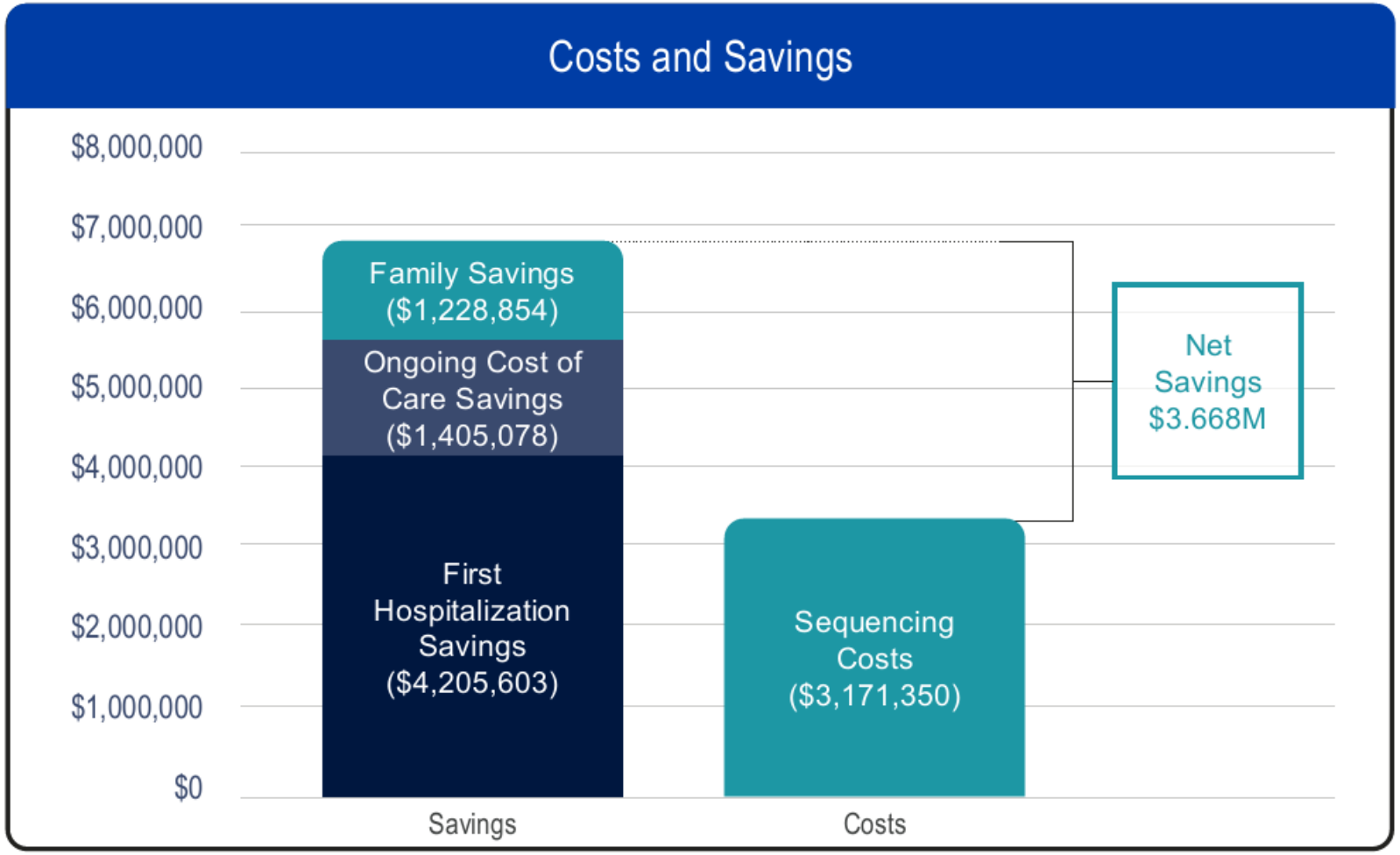
** Results confirmed 21 babies were already receiving appropriate care*
*** Median # days to delivery of provisional positive results*

project baby bear

Medicaid: Federal-paid State-administered insurance plan that covers 55% of NICU patients

Am J Hum Genet. 2021 108:1231-1238.

It took Cost Effectiveness studies to achieve Medicaid coverage of rWGS in Neonatal Intensive Care Units



Am J Hum Genet. 2021 108:1231-1238.

~250,000 Medi-Cal covered infants born per year

2,500 infants receiving rWGS®

Effective change in treatment

758.5

QALYS gained

$28m

Net Savings

Evidence base for rWGS in 2024

- 37% diagnosis
- 26% change in management
- 18% change in outcome
- $17,243 median net savings per test

Ref.	Year	Country	Number of probands	Dx rate	Net savings per proband
48	2018	USA	42	43%	$18,741
68	2021	USA	184	40%	$6,294
6	2022	USA	61	33%	$11,286
85	2022	USA	38	45%	($1,436)
75	2022	USA	65	40%	$100,440
86	2022	Australia	40	53%	$17,243
77	2023	USA	89	39%	$4,155
87	2025	USA	184	40%	$22,396
78	2025	USA	400	49%	$158,592
		Median		**40%**	**$17,243**

npj Genomic Med. (2024) 9:17 (2024)

Ref.	Year	Country	Study Type	Test	Enrollment Criteria	Size	Dx Rate	Δ Mx	Δ Outcome	TAT (days)
7	2012	USA	Cases	URGS	NICU infants; Susp. genetic disease	4	75%	n.d.	n.d.	2
44	2015	USA	Cohort	RGS	<4 months of age; Susp. actionable genetic disease	35	57%	31%	29%	23
45	2017	USA	Cohort	RES	<100 days old; Susp. genetic disease	63	51%	37%	19%	13
46	2017	Holland	Cohort	RGS	Infants; NICU, PICU; Susp. genetic disease	23	30%	22%	22%	12
47	2018	USA	RCT	RGS,SOC	<4 months of age; Susp. genetic disease	32	41%	31%	n.d.	13
48	2018	USA	Cohort	RGS	Infants; Susp. genetic disease	42	43%	31%	26%	23
49	2018	Aust	Cohort	RES	Acutely ill children with susp. genetic disease	40	53%	30%	8%	16
50	2018	UK	Cohort	RGS	Children; PICU and Cardiovascular ICU	24	42%	13%	n.d.	9
51	2019	USA	Cohort	RGS	4 months-18 years; PICU; Susp. genetic disease	38	48%	39%	8%	14
52	2019	UK	Cohort	RGS	Susp. genetic disease	195	21%	13%	n.d.	21
12	2019	USA	Cases	URGS	Infants; ICU; Susp. genetic disease	7	43%	43%	n.d.	0.8
53	2020	USA	Cohort	RES	<6 months old; ICU; hypotonia, seizures, metabolic, multiple congenital anomalies	50	58%	48%	n.d.	5
54	2019	Canada	Cohort	RES	NICU; infants; susp. genetic disease	25	72%	60%	n.d.	7.2
55	2019	Taiwan	Cohort	RES	PICU and other; children; susp. genetic disease	40	53%	43%	n.d.	6
56	2020	China	Cohort	RES	NICU & PICU; complex	130	48%	23%	n.d.	3.8
57	2020	USA	Cohort	RES	Critical illness; medical genetics selected	46	43%	52%	n.d.	9
58	2020	USA	Cohort	RES	PICU; < 6 years; new metabolic/neurologic disease	10	50%	30%	n.d.	9.8
59	2020	USA	Cohort	RES	ICU; infants	368	27%	n.d.	n.d.	n.d.
60	2020	China	Cohort	RES	Infants; ICU and inpatient	102	31%	27%	n.d.	11
61	2020	USA	Cohort	RES	Various	41	32%	n.d.	n.d.	7
62	2020	Aust	Implem	URES	<18 year; NICU and PICU	108	51%	44%	n.d.	3
63	2020	Poland	Cohort	RES	Infants; NICU, PICU; susp. genetic disease	18	83%	61%	n.d.	14
64	2020	China	Cohort	URES	Infants; NICU, PICU; susp. genetic disease	33	70%	30%	30%	1
33,65, 66	2019, 2020	USA	RCT	RGS	Infants; disease of unknown etiology; within 96 hours of admission	94	19%	24%	10%	11
				RES		95	20%	20%	18%	11
				URGS		24	46%	63%	25%	4.6
68	2021	USA	Implem	URGS	Medicaid infants; unknown etiology; within 1 week c	184	40%	32%	n.d.	3
69	2021	China	Cohort	RES	Critically ill; 6 days - 15 years; susp. genetic disease	40	43%	31%	n.d.	5
70	2021	Germany	Cohort	RES	NICU, PICU, infants; sup. Genetic disease	61	43%	11%	n.d.	60
71	2021	USA	RTDCT	RGS,WGS	<120 days old; ICU; susp. genetic disease	354	31%	25%	n.d.	15
43	2021	China	Crossover	RES	Critically ill infants with conditions suggestive of genetically heterogeneous disorders	202	20%	n.d.	n.d.	20
				RGS		202	37%	7%	n.d.	7
72	2022	France	Cohort	RGS	NICU, PICU with probable genetic disease; urgent ne	37	57%	n.d.	n.d.	43
73	2022	UAE	Cohort	URGS	Infants in ICU with complex multisystem disease	5	60%	20%	20%	1.5
74	2022	USA	Implem	RES	NICU infants with susp. genetic disease	80	28%	18%	n.d.	13
75	2022	USA	Cohort	RGS	Children in ICU with disease of unknown etiology	65	40%	n.d.	n.d.	12
76	2022	France	Cohort	RES	Infants in ICU with susp. genetic disease	15	40%	53%	n.d.	16
77	2023	USA	Implem	RGS	NICU, PICU with disease of unknown etiology	89	39%	27%	n.d.	n.d.
67,78	2021, 2023	USA	Crossover	RGS, panel	NICU with disease of unknown etiology	400	49%	19%	n.d.	6
79	2023	USA	Cohort	RGS	Acutely ill inpatient infants; susp. genetic disease	188	35%	32%	n.d.	6
80	2023	Belgium	Cohort	URGS	NICU, PICU, neurologic inpatients with susp. genetic	21	57%	57%	n.d.	1
Weighted Average						**3609**	**37%**	**26%**	**18%**	

Current Medicaid Coverage of rWGS

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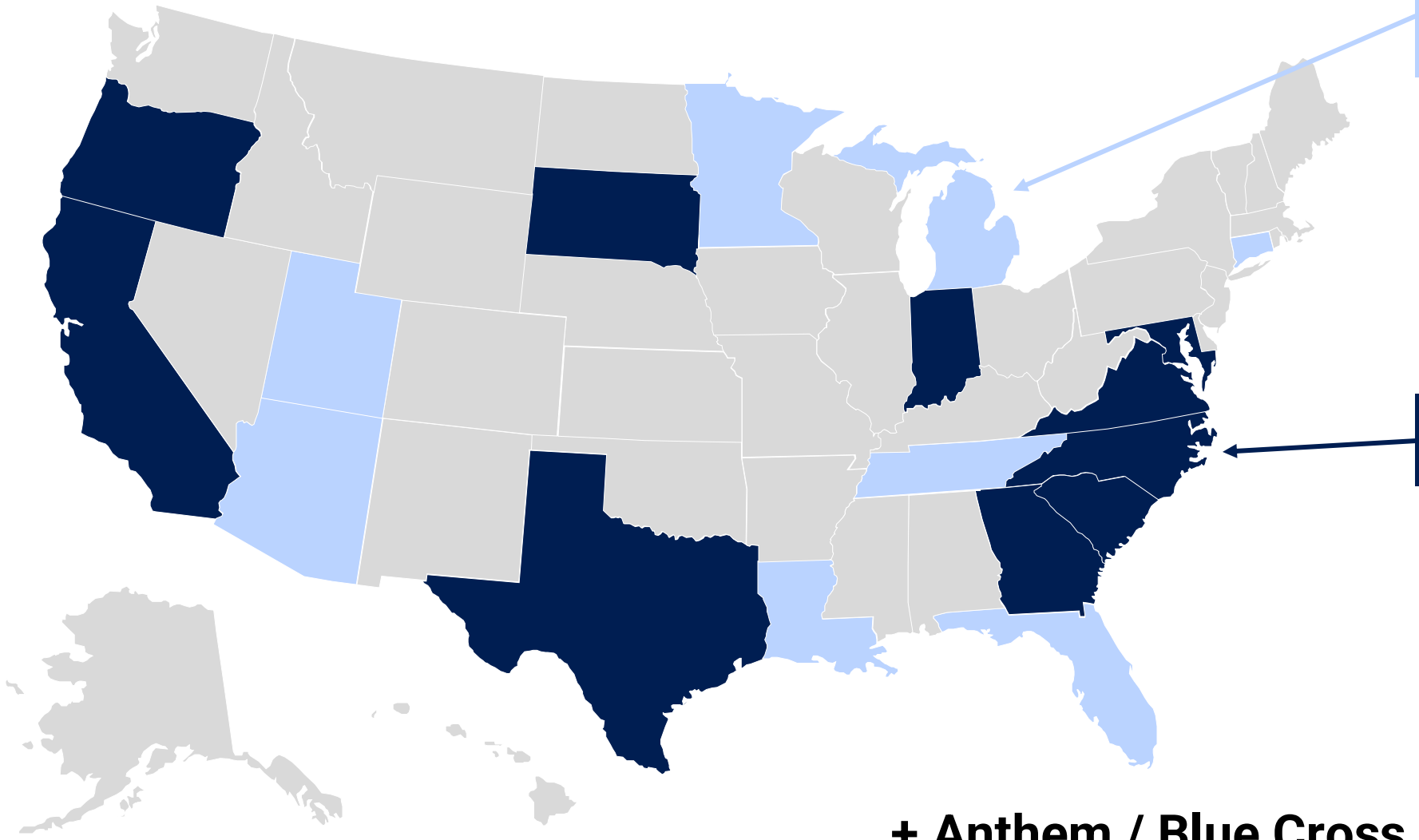

8 with Separate Payment
Utah, Arizona, Louisiana, Florida, Tennessee, Connecticut, Michigan, Minnesota

10 with No Separate Payment
Diagnosis-related bundled payment

+ Anthem / Blue Cross / Blue Shield nationwide

Complex Coverage Policies for rWGS

1. Sx suggest a genetic disease that cannot be Dx by standard work-up;
2. Sx suggest a broad, differential Dx that requires multiple genetic tests;
3. Timely Dx necessary to guide clinical decision making; and >1 of the following:
    a. Multiple congenital anomalies,
    b. Specific malformations suggest a genetic disease,
    c. Laboratory test suggests a genetic disease,
    d. Refractory or severe hypoglycemia,
    e. Abnormal response to therapy,
    f. Severe hypotonia,
    g. Refractory seizures,
    h. Brief Resolved Unexplained Event:
    i. Laboratory test suggests inborn error of metabolism,
    j. Test suggests channelopathy, arrhythmia, cardiomyopathy, myocarditis, or structural heart disease,
    k. Family history of genetic disease related to symptoms/signs.

npj Genomic Med 9:17 (2024)

After 13 years, US Adoption of rWGS is ~4% of those in need

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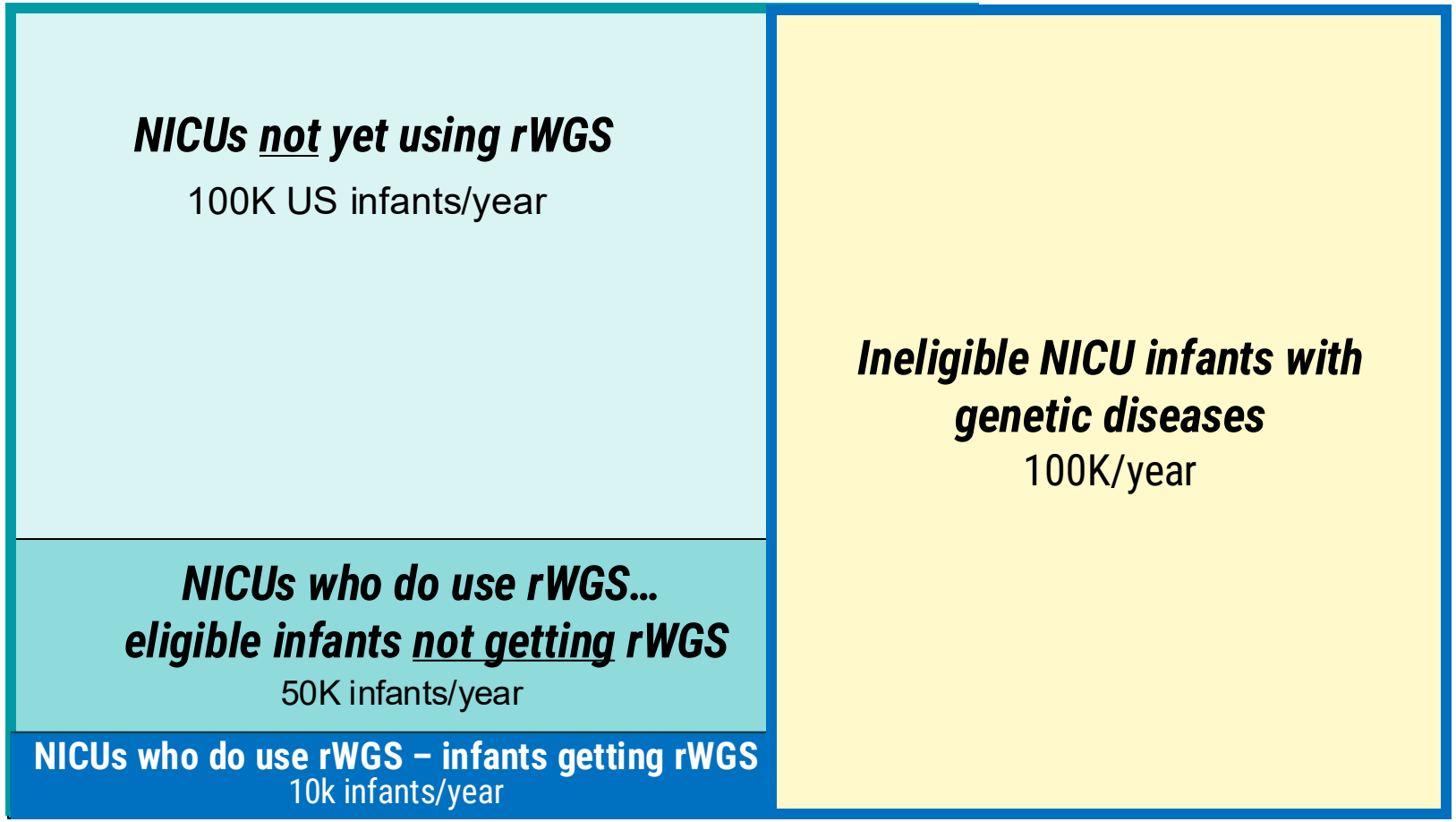
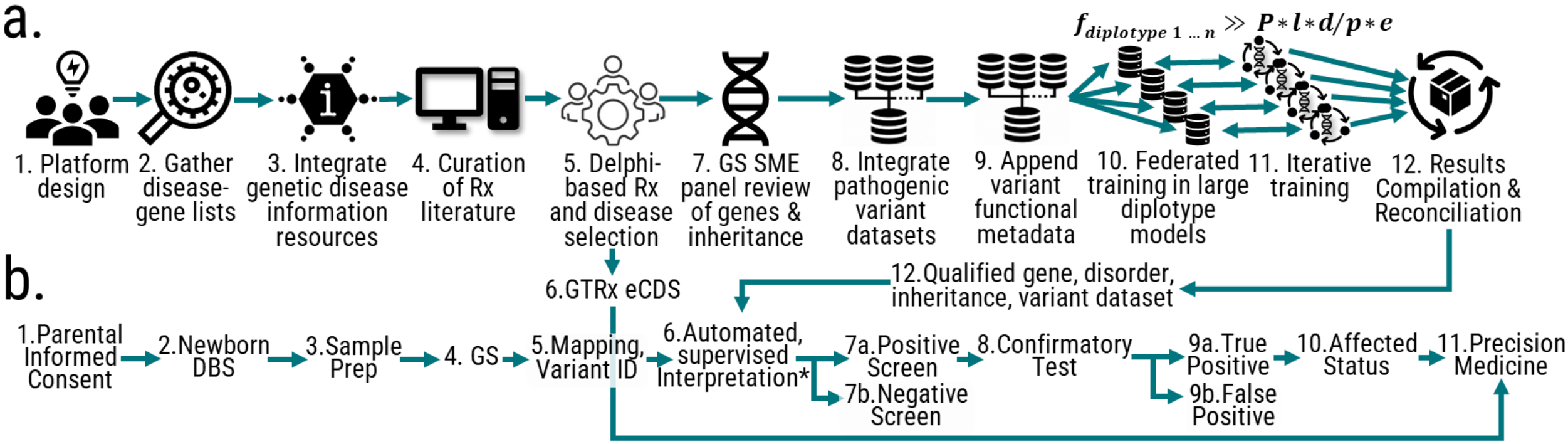


Re-engineering rWGS for BeginNGS NBSxWGS



Version 3 BeginNGS Platform

510 severe, early childhood diseases with effective therapies

Am J Hum Genet. 2022 109:1605-1619 and 2024 111:2618-2642.

Ref	Design	Seq Type	Size	Subjects	Disorders	Genes	Screen +ve	Sensitivity	Specificity	Clinical Utility
8	Retro	WGS	1,696	Infants	n.d.	163	2.0%	94%	63%	n.d.
9	Retro	WES	1,190	NB	48	78	n.d.	88%	98%	n.d.
10	Pro	WES	106	NB	n.d.	466	n.d.	44%	n.d.	n.d.
11	Pro	Panel	1,127	NB	n.d.	463	0.5%	n.d.	100%	n.d.
12										
13										
14										
15										
16										
16										
17										
18										
18	Retro	WES	606	Children	n.d.	268	2.1%	n.d.	n.d.	n.d.
19	Pro	Panel	29,601	NB	128	142	2.7%	82%	50%	n.d.
20	Pro	Panel	10,220	NB	94	164	2.4%	n.d.	23%	n.d.
21	Pro	WES	3,423	NB	542	601	11.4%	59%	44%	n.d.
21	Retro	WGS	301	Adults	542	601	7.6%	n.d.	n.d.	n.d.
22	Pro	WGS	3,982	NB	255	237	3.7%	n.d.	79%	n.d.
23	Pro	WES	7,000	NB	n.d.	2350	0.9%	n.d.	n.d.	n.d.
24	Retro	WGS	3,118	ICU children	412	342	7.2%	99%	98%	n.d.
24	Retro	WGS	705	Infant deaths	412	342	8.7%	n.d.	90%	5.3%
24	Retro	WGS	3,519	Parents	412	342	3.6%	n.d.	n.d.	n.d.
24	Retro	WES	469,902	Adults	412	342	2.0%	n.d.	n.d.	n.d.
25	Pro	WGS	120	ICU NB	412	342	4.2%	83%	100%	4.2%
25	Pro	WGS	120	ICU NB	n.d.	2000	10.8%	93%	100%	9.9%
26	Pro	WES	3,847	NB	165	405	1.8%	n.d.	98%	1.6%

- 19 studies
- 48 – 6000 disorders
- Clinical utility examined in 6 studies: 1.6 – 9.9%
- Use in ICUs seems worthwhile

Summary

Rady Children's Institute Genomic Medicine®

1. Rapid genome sequencing (rWGS) provides timely diagnosis for (almost) all genetic diseases
2. rWGS improves outcomes and lowers cost in NICUs and PICUs
3. rWGS has been implemented in <5% of those who need it
4. Screening (NBS) WGS is complementary to rWGS; together they provide a foundation for genome-informed healthcare; more evidence is needed to substantiate this
5. There are major technological challenges to NBSxWGS that require AI-based interpretation process with training from very large genome datasets
6. Democratization of rWGS and NBSxWGS requires engagement of stakeholders, parents, and physicians and upskilling of physicians

NEWBORN SCREENING
Wins & Losses

Lynsey Chediak
Associate Director, State Government Affairs, US West
BioMarin

BIOMARIN®

Topics For Discussion

- Reviewing what types of NBS-related legislation were introduced
- ACHDNC dissolution
- 2025 “Wins”

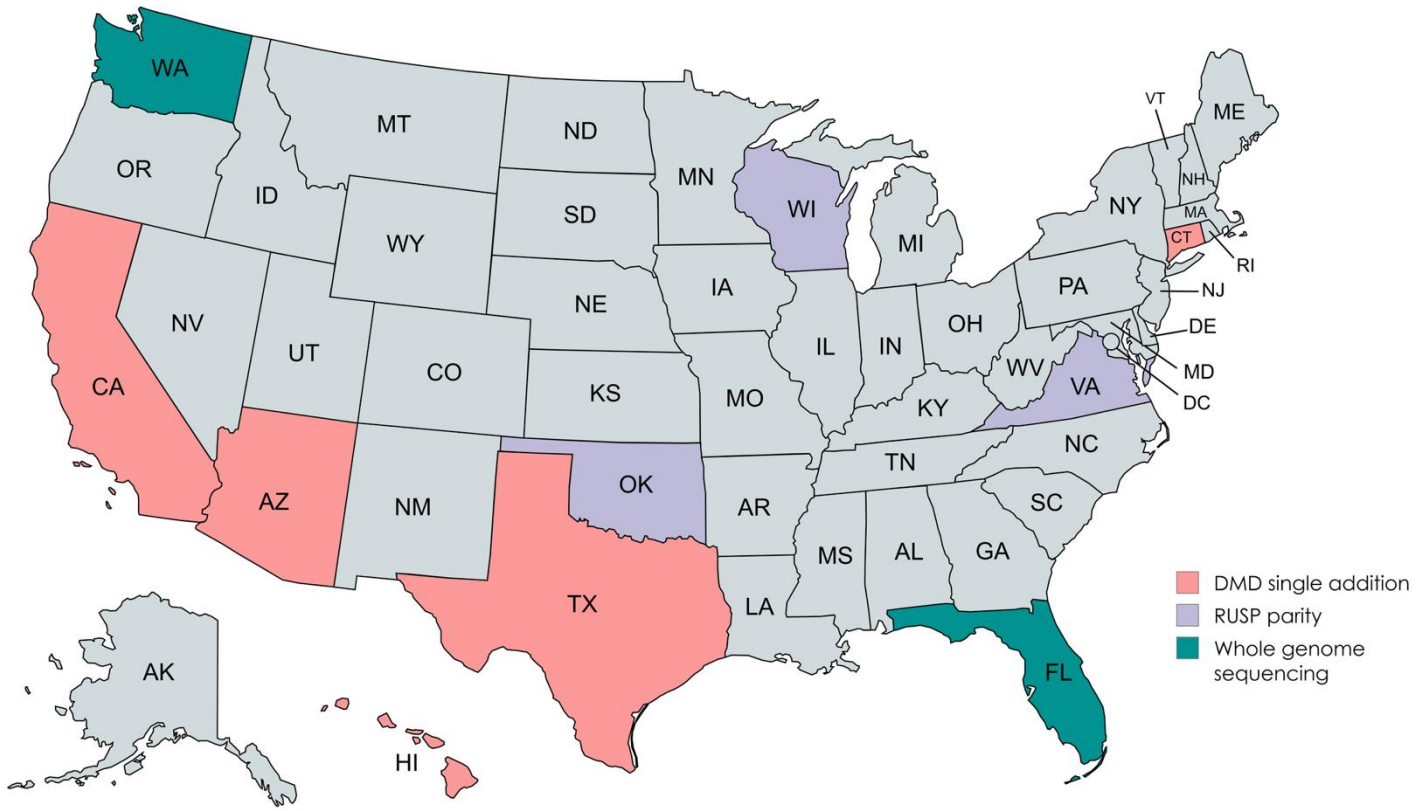
RARE READY
A GENETIC CONDITION COALITION

Expanding NBS Bill Introductions

Types of bills

- Single Condition Addition – Duchenne Muscular Dystrophy (DMD)
- RUSP Parity
- NBS Funding Expansion (state specific)

State Legislators Demonstrated Interest in NBS Expansion



*Some states had multiple NBS bills covering more than one topic highlighted above

**Not an exhaustive list

RUSP Parity and Federal Administration Changes

Health Resources & Services Administration

HRSA Health Resources & Services Administration | Federal Advisory Committees

- HRSA Advisory Committee formed in 2003 called the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC).
- The ACHDNC recommended its first panel of conditions called the Recommended Uniform Screening Panel (RUSP) in 2010.
- RUSP conditions are selected based on:
  - Identifiable conditions within 1-2 days of birth
  - Available, validated screening test
  - Newborn benefit from early detection and intervention
  - Available FDA-approved treatment
- 64 rare conditions on the RUSP.
- ACHDNC dissolved in April 2025 by the Department of Health and Human Services (HHS) as part of a broader restructuring effort.
- HHS Secretary Robert F Kennedy can technically add conditions to the RUSP (MLD and DMD) – federal register comment period ended Sept 15, 2025.

Highlights of 2025 Legislative Action

DMD Added

- Texas
- Arizona
- Florida

Lab Funding Added

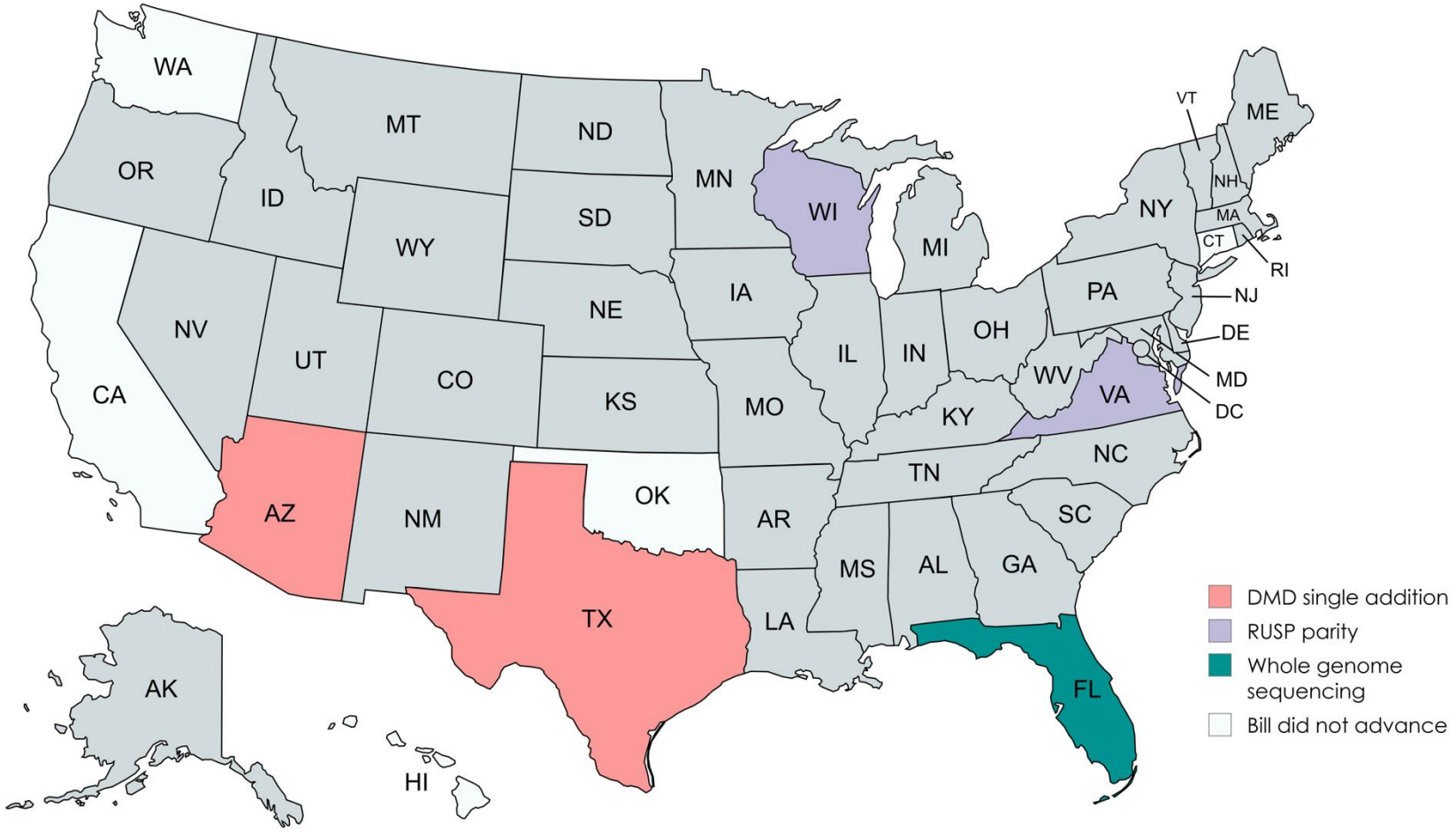
- Texas
- Arizona

RUSP Alignment

- Virginia
- Wisconsin (still in active session)

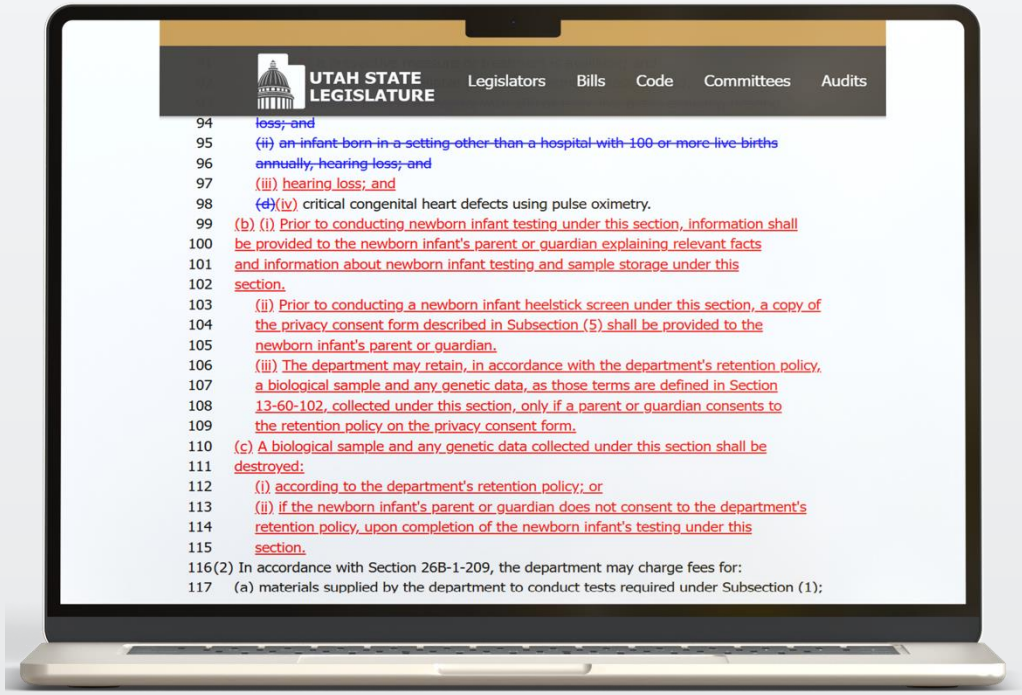
rWGS Expansion

- Florida



Utah Law on Parental Consent for Newborn Genetic Data

- Utah Governor Spencer Cox signed HB 363 (Rep Pierucci) into law.
- Requires parental consent to retain a newborn's blood sample or genetic data after the initial newborn screening.



Legislative Outlook for 2026 Sessions

- Continued interest in adding DMD through legislative action.
- Further single condition addition bills.
- Continued need for state lab funding to grow as populations expand (state specific).
- RUSP uncertainty.

Questions

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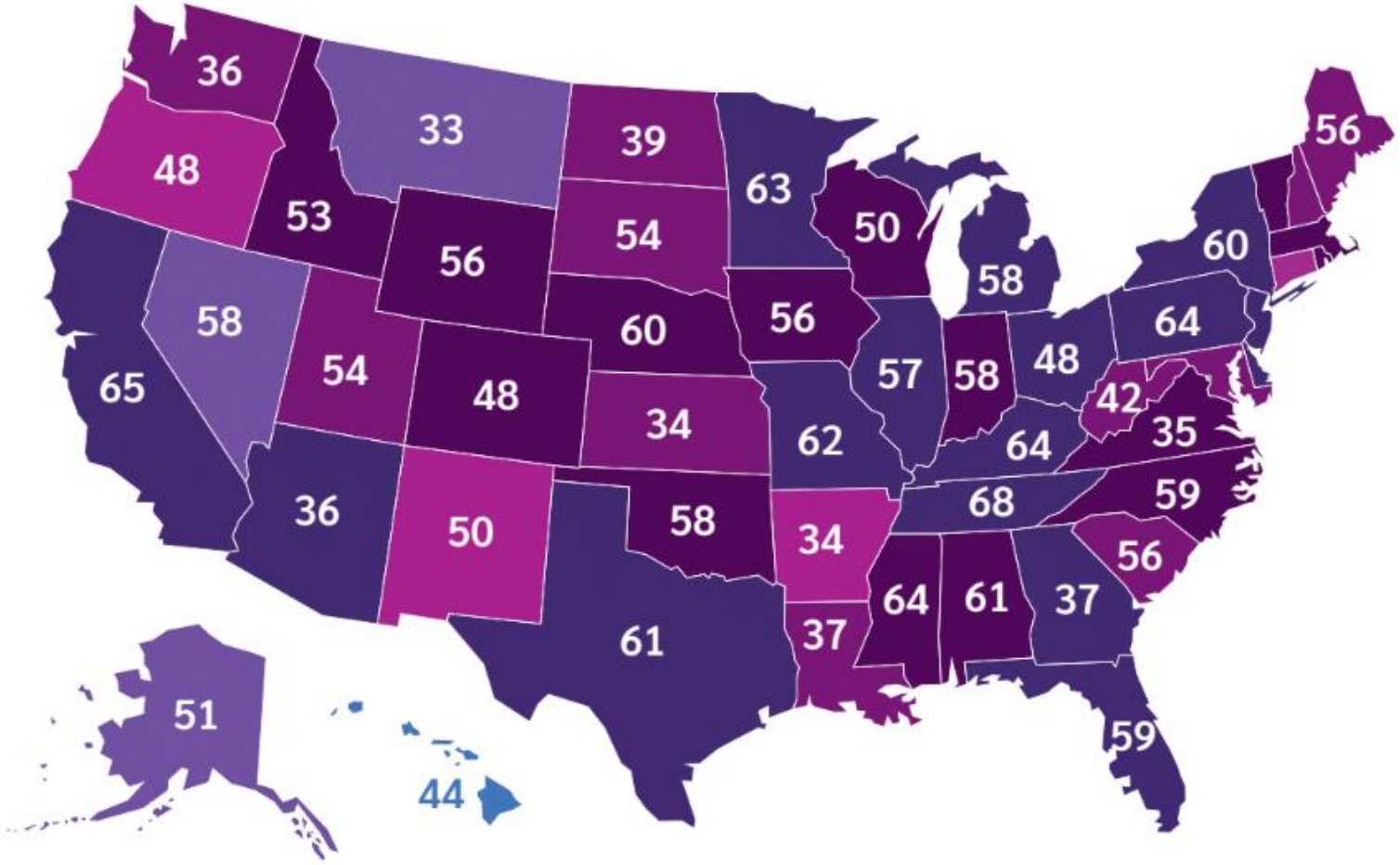
NEWBORN SCREENING

Looking Ahead

Erin Frey
Senior Director State Government Affairs
Ultragenyx

ultragenyx

The State of Newborn Screening



MA	66
RI	35
NJ	63
MD	61
DE	58
NH	42
CT	63
VT	35

DC	63
Puerto Rico	52
Guam	39
Virgin Islands	34

16	States screen for 36+ core conditions
15	States screen for 35 core conditions
11	States screen for 34 core conditions
4	States screen for 33 core conditions
3	States screen for 32 core conditions
1	States screen for 31 core conditions

Numbers in each state represent the total number of conditions screened for in each state.

Source: babysfirsttest.org
Data as of September 4, 2025.

Understanding the Landscape of Newborn Screening

BACKGROUND

- Over 10,000 rare diseases affect 1 in 10 Americans, yet only 5% have approved treatments.
- Early diagnosis is critical—many rare disease therapies, including gene therapies, are most effective when administered before symptoms appear.
- NBS is a proven public health tool that enables early detection of treatable conditions using a simple heel-prick blood test.
- NBS panels are determined at the state level, typically aligned with the RUSP, a federally curated list of conditions.
- Adding a condition to NBS involves a multi-year process of federal and state reviews, followed by lab validation to ensure accuracy and reliability, typically taking 5-10 years after a treatment is approved.

CURRENT CHALLENGES

- The termination of the federal ACHDNC has disrupted the RUSP nomination and approval process.
- Conditions like MLD and DMD were the last to be reviewed by ACHDNC and are awaiting US DHHS Secretary's decision whether to add to the RUSP, but how conditions will be added is uncertain.
- Many treatable, screenable conditions remain un-nominated due to the complexity and cost of the process.
- State NBS labs are under-resourced, facing staffing and infrastructure challenges that hinder timely expansion.
- Without proactive state action, babies with treatable conditions may go undiagnosed, missing critical windows for intervention.

In the absence of ACHDNC, what are things that can be considered by states and public health labs to continue to support the rare community?

Priority #1: Keep NBS Top of Mind

Recommended Actions for State Legislators

- Advance legislation to authorize condition additions and appropriate funding—this positions the state to act swiftly when federal processes resume.
- Establish or expand dedicated NBS funds and consider adjusting screening fees to support lab capacity and sustainability.
- Prioritize conditions with available treatments (and/or available clinical trials) and existing screening technology.
- Enable labs to maximize use of existing technology—many panels already detect more conditions than are reported because labs cannot report results unless officially added to the state panel.
- Assess NBS Advisory Committees' review processes to seek efficiencies and reduce time.
- Consider state Rare Disease Advisory Council support, advice and coordination.

Recommended Actions for Public Health Lab Leaders

- Lab leaders' insights are essential—share operational challenges and recommendations to inform policy and funding decisions.
- Advocate for flexible funding mechanisms, such as screening fee adjustments, to cover direct and indirect costs (i.e. staffing, IT, equipment, facilities, validation).
- Recognize that federal delays may persist—states must act independently to ensure babies are not left behind.
- Even with RUSP alignment laws, state-level review and validation remain necessary for each condition—early planning is key.
- Maintain a list of conditions that have 1) approved treatments and 2) a NBS assay and add these conditions to state screening panels as appropriate.
- Consider state Rare Disease Advisory Council support, advice and coordination.

Ecosystem Considerations for State NBS

PROS

- Conversation about NBS **will continue.**
- Learnings could inform federal level outcomes.
- NBS is a **bipartisan issue.**
- **Mobilizes legislators** to act on a proven public health program.
- Legislators are compelled by adding multiple conditions (not any one company's self interest).
- Prevents diagnostic odyssey and **improves health outcomes**.

NEUTRAL

- This could be a **multi-year effort.**
- The effort to reinstate or reinvent the **ACHDNC will be happening simultaneously.**
- Each state, if they engage, will have their own **unique path.**
- This effort is **horizon scanning.**
- **Identifying milestones** that would trigger funding will support PHLs.

CONS

- This is a complex **course change** from RUSP Parity legislation.
- NBS Advisory Committees will still review conditions on their timelines with their unique processes.
  - All NBS Ad Comms require evidence, NBS program readiness (physician ed & follow up plans), treatment availability, and a very specific and sensitive assay that has VERY low False Positives and zero False Negatives.
- PHLs tend to perceive legislation as pressure.

Polling Question

Are there stories from your community that could help educate legislators or public health leaders about the importance of newborn screening?

NEWBORN SCREENING
ADVOCACY

Kari Lato
Sr. Director, Policy & Advocacy
Rx4good

Strategic Planning for Legislation Introduction

What it is

- State-level law requiring NBS programs to add a specific condition.
- Creates a legal mandate with a deadline.
- Speeds up access to life-saving screening.
- Introduced as a bill, goes through hearings and votes, signed by governor.
- Has been used to add conditions like DMD disease.

Why it's important

- Saves lives by ensuring earlier diagnosis and treatment.
- Prevents diagnostic odyssey and improves health outcomes.
- Mobilizes legislators to act on a proven public health intervention.
- Strengthens the state's public health infrastructure.

What we will do

- Identify states where legislation has a chance.
- Consider political climate, budget implications, and timing in the legislative session.
- Find a legislative champion and draft bill.
- Secure Senate and House cosponsors to ensure the bill moves in both chambers and has bipartisan backing.
- Introduce the bill at the optimal time in the session.
- Ask for your help once the bill is introduced!

How You Can Help When Legislation is Introduced

There will be opportunities to:

- Testify in person or submit letters of support.
- Meet with legislators (1:1, small groups, hill days).
- Call your legislators.
- Lend your logo to group letters.
- Use social media to educate and bring awareness to the issue – make sure to tag legislators and include relevant hashtags.
- Mobilize your constituents to take action.
- Identify patients/families willing to share their stories (with legislators, media, public via social/blog).
- Identify HCPs / experts to participate in meetings.

Polling Question

After attending this webinar, how would you rate your understanding of newborn screening compared to before?

- **Significantly improved** – I feel much more informed and confident in my understanding.
- **Somewhat improved** – I have a better grasp but still have some questions.
- **About the same** – My understanding hasn't changed much.
- **Still unclear** – I need more information to fully understand.

THANK YOU

JOIN THE COALITION TODAY!

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RARE READY COALITION