

The Importance of a Long-term Follow-up Program for SCID

Morna J Dorsey, MD, MMSc

Immune Deficiency Foundation: SCID Compass

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Agenda



- The SCID Journey
- Long-term follow-up questions
- *Ca/SCID* Program
 - Needs assessment
 - Health-specific outcomes
 - Data sharing
 - Education
- Conclusion

The SCID Journey Map



What is Long-term Follow-up?

HRSA: “treatment and management following diagnosis and referral”

- There is no definitive way to conduct LTFU for SCID
- It is not entirely clear what LTFU should entail
 - Most centers may have similar protocols for early management
 - No SCID-specific consensus pathway for follow up beyond definitive therapy



Why is Long-term Follow-up important?

- SCID is heterogenous
- SCID is a rare condition that requires long-term follow-up
- Care is received in diverse settings
- Lack of natural history studies
- Incomplete understanding of how factors such as treatment pathways and type of SCID impact long-term outcomes

Your Questions

What are the short-term complications post-treatment that parents should be aware of?

What are the long-term complications post-treatment?

Will a child need special services in school?

Are a child's LTFU needs different based on the type of SCID they have or treatment they received?

What is the likelihood that a patient will need additional treatment when they are older?

How often should a child check in with their immunologist?

Research Focus

Long-term outcomes

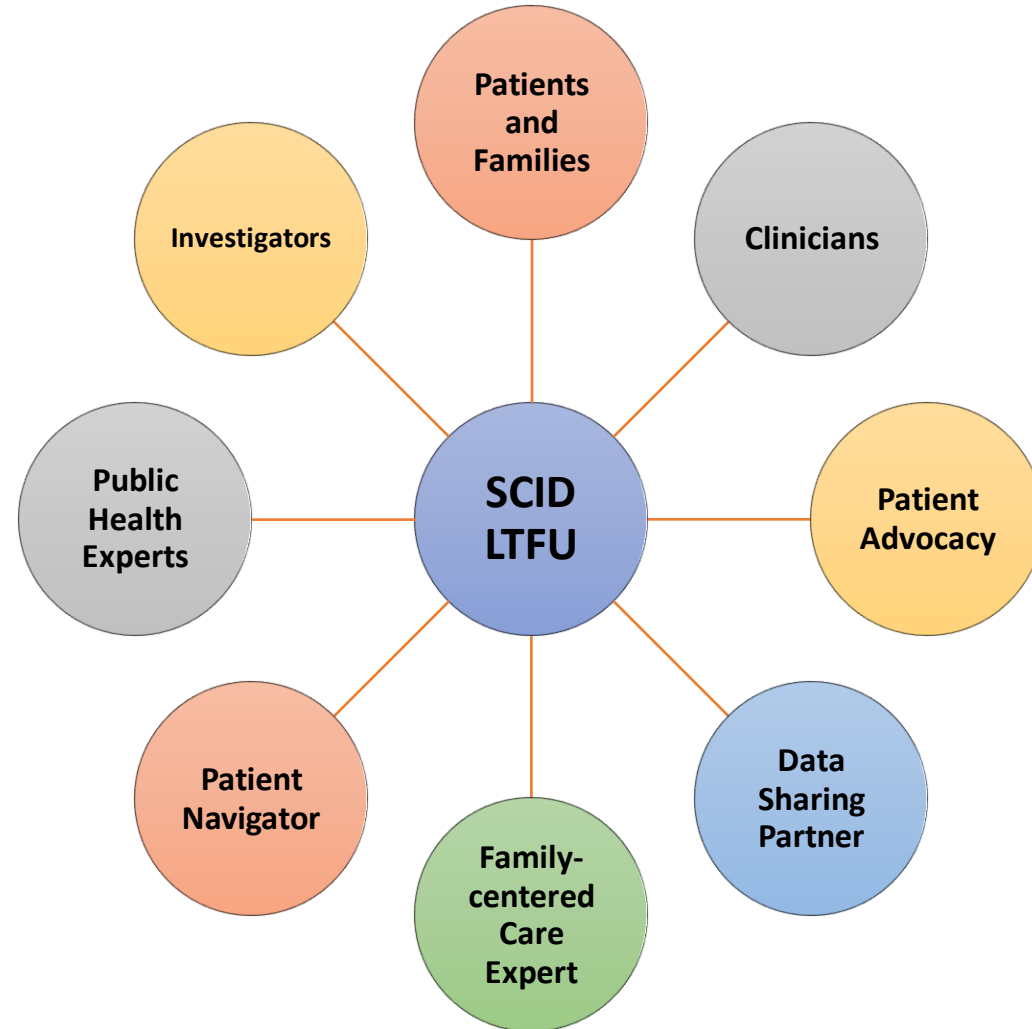
Neurodevelopment and school readiness

Health-specific services needed

Transition of care

Family well-being

Long-term Follow-up Program: A Stakeholder Approach





California Severe Combined Immunodeficiency Consortium Long-term Follow-up

Sites:

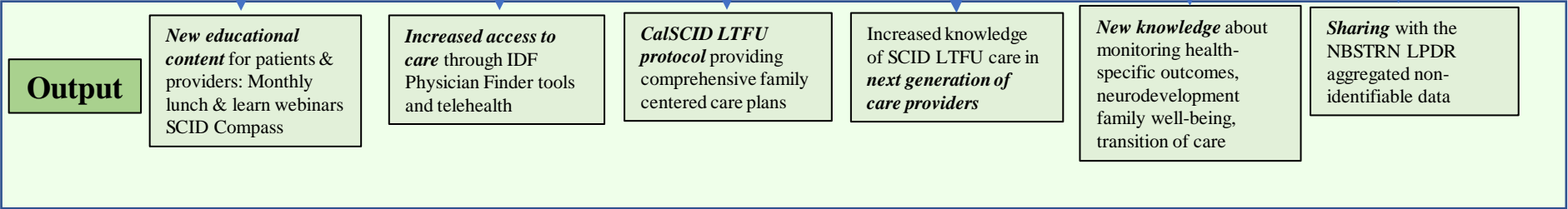
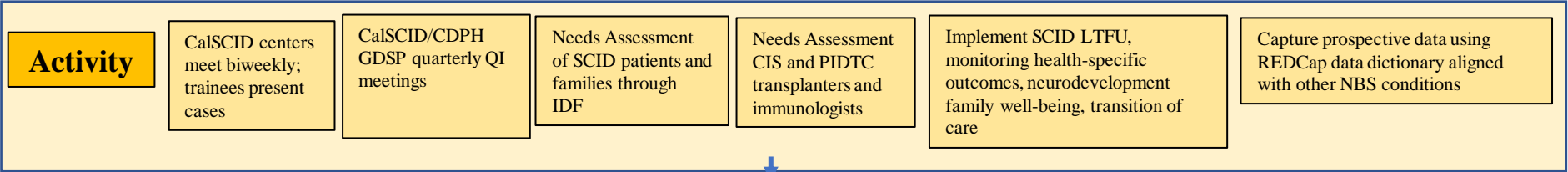
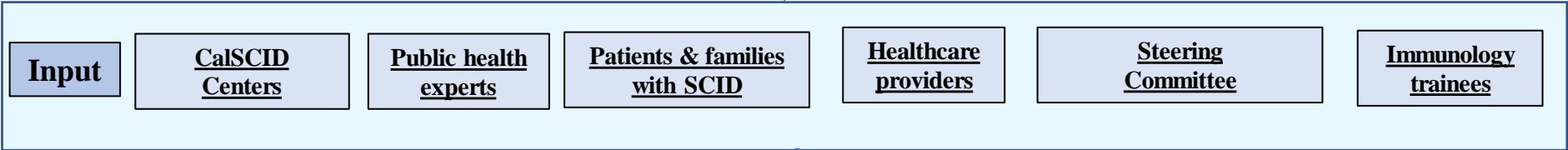
- University of California San Francisco
- University of California Los Angeles
- University of California Davis
- University of California Irvine
- University of California San Diego
- Stanford

Partners:

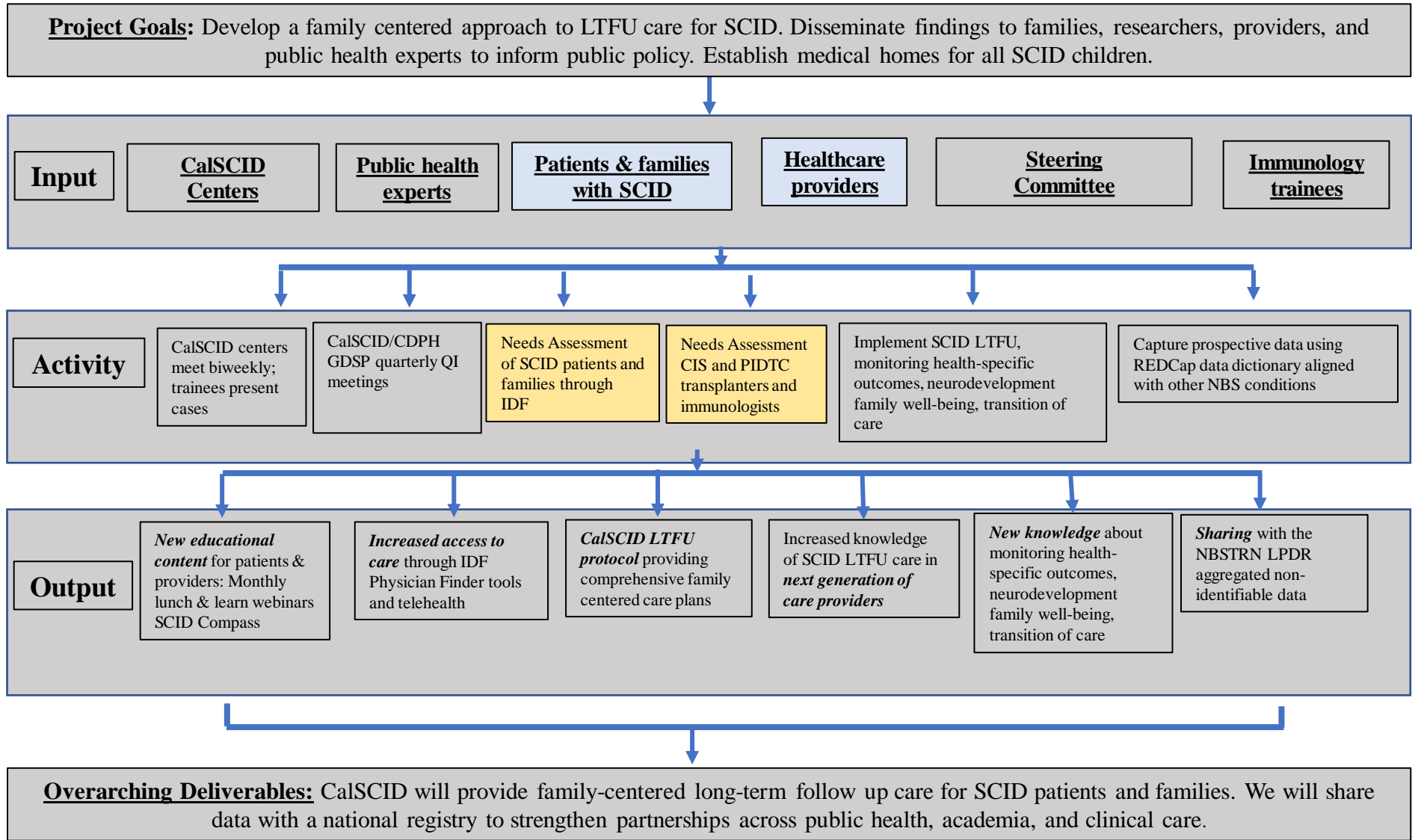
- California Department of Public Health
- Immune Deficiency Foundation
- Primary Immune Deficiency Treatment Consortium
- SCID, Angels for Life
- Newborn Screen Translational Research Network



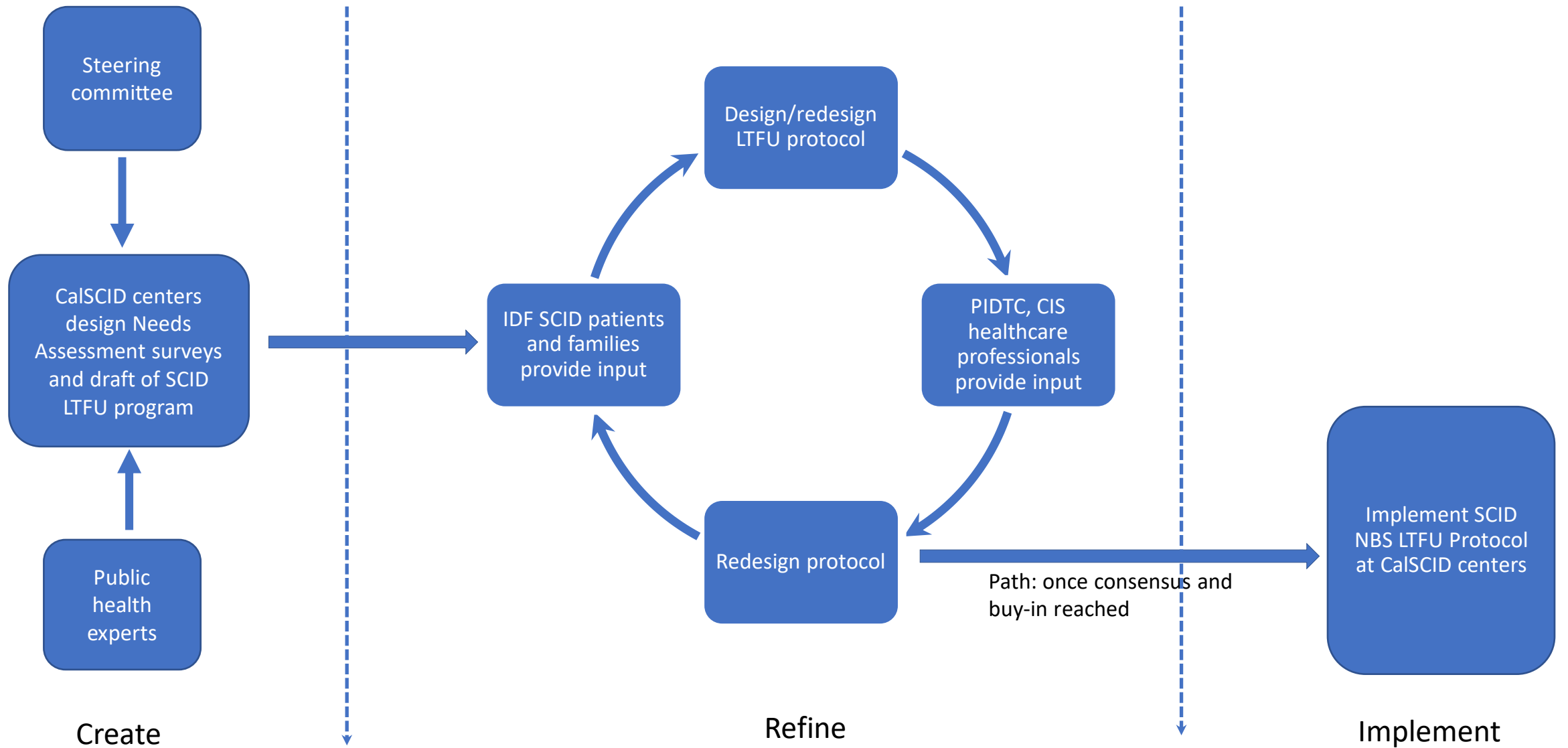
Project Goals: Develop a family centered approach to LTFU care for SCID. Disseminate findings to families, researchers, providers, and public health experts to inform public policy. Establish medical homes for all SCID children.



Overarching Deliverables: CalSCID will provide family-centered long-term follow up care for SCID patients and families. We will share data with a national registry to strengthen partnerships across public health, academia, and clinical care.



Overview of development of SCID LTFU program

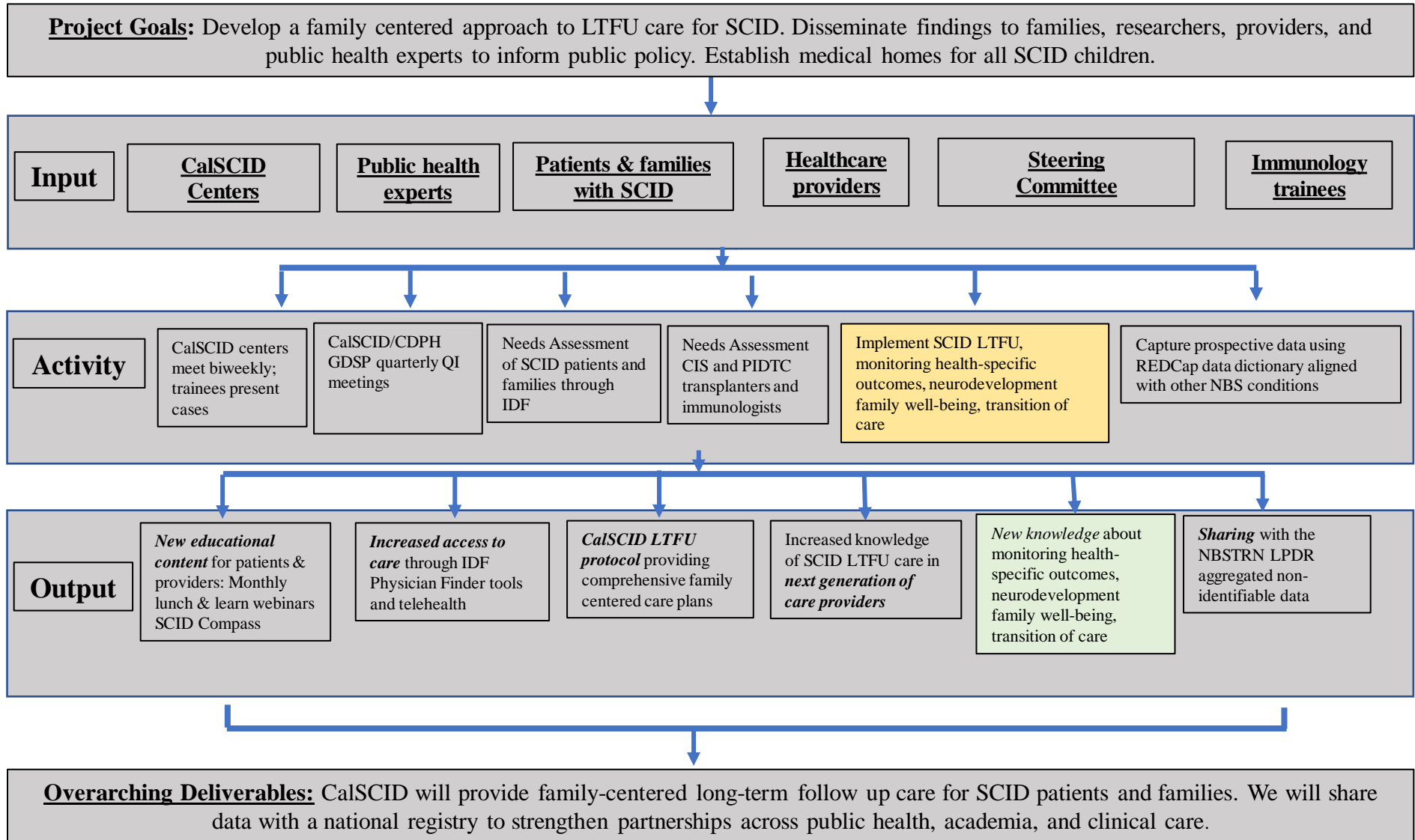


Patients and Families

- Demographics
- Information support
- Emotional support
- Financial support
- Development
- Medical Home
- Access to care
- Transition of care

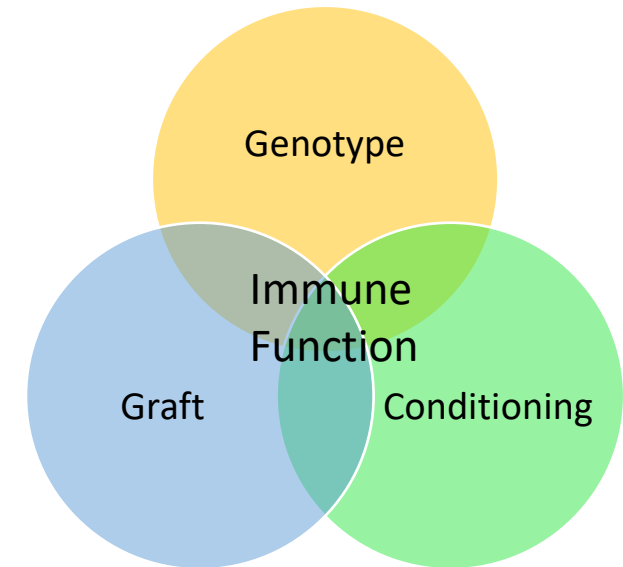
Healthcare Providers

- Demographics
- Current practices
- Medical home
- Transition of care
- Barriers
- Development assessment
- Wellbeing assessment
- Information needs



Health-specific Outcomes: Immune Function

- To date, focus has been on survival and not about outcomes beyond survival
- Durability of Immune Reconstitution
- Impact of genotype and on non-immune organ system
- Limitations of transplant on non-hematopoietic immune function
- Frequency and type of immune evaluation



Health-specific Outcomes: Beyond Immune Reconstitution

- Allergy
- Audiology
- Cardiology
- Dental/oral medicine
- Dermatology
- Endocrinology
- ENT
- Gastroenterology
- General Care
- Genetics
- Hematology/Oncology
- Infectious Disease
- Nephrology
- Neurology
- Nutrition/ Feeding Team
- Ophthalmology
- Pulmonary Medicine
- Psychology
- Psychiatry
- PT/OT
- Social Work
- Surgery
- Rheumatology

Health-specific Outcome: Neurodevelopment

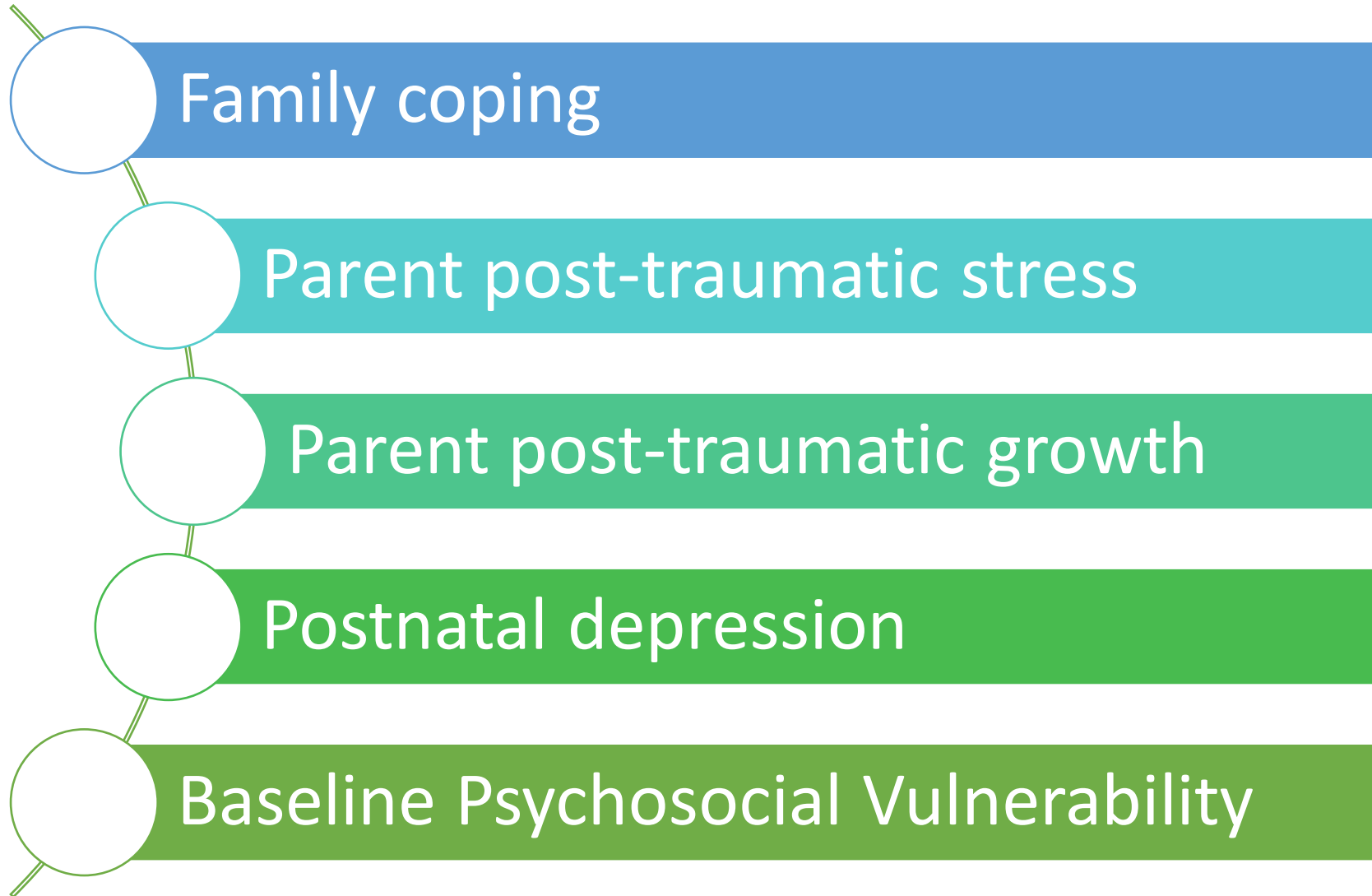
- Paucity of literature on outcomes following HCT and the impact on neurocognitive development in different SCID genotypes and conditioning regimens
- Different depending on type of SCID
- Different depending on type of conditioning
- Ideal: Formal assessment of neurodevelopment at 5 years
- School readiness

Health-specific Outcome: Family Wellbeing

- Understand stressors
- Uncover ways to lessen psychological trauma and promote wellbeing
- Understand vulnerabilities in our healthcare delivery
- Examine resilience



Parent and
Family
Wellbeing
Assessment





Adapted from Getty
Images

Personalized Communication



About me

I was so happy to be pregnant again after a previous miscarriage. It's hard for me to understand how my baby could have SCID because she looks so perfect.. I've read a lot about SCID and I know that my baby is likely to survive but I don't know what the future looks like and if there are cases of babies who don't survive. My faith will help me get through this.

Melissa

Information Seeking Mother

Age: 34

Occupation: Executive Assistant

Education: Bachelor's Degree

Relationship: Married

Children: 3 year-old daughter

Support: Husband, and mother-in law

"...we didn't actually find out how long we might be here until [later in the hospitalization]...I think that was pretty tough. I think doctors...don't want to deliver kind of blow by blow, but—I think it's harder to find out later than to know upfront."

Currently I feel....

Overwhelmed, guilty, isolated, sad

Goals

Wants family to be healthy and happy

Wants to raise kids that have the best opportunity

Wants to be more environmentally conscious

How to communicate with me

- ✓ Give me straight answers
- ✓ Give me details
- ✓ Provide interactive educational materials
- ✓ Use images to illustrate information
- ✓ Clearly describe what to anticipate week-by-week


How I get information

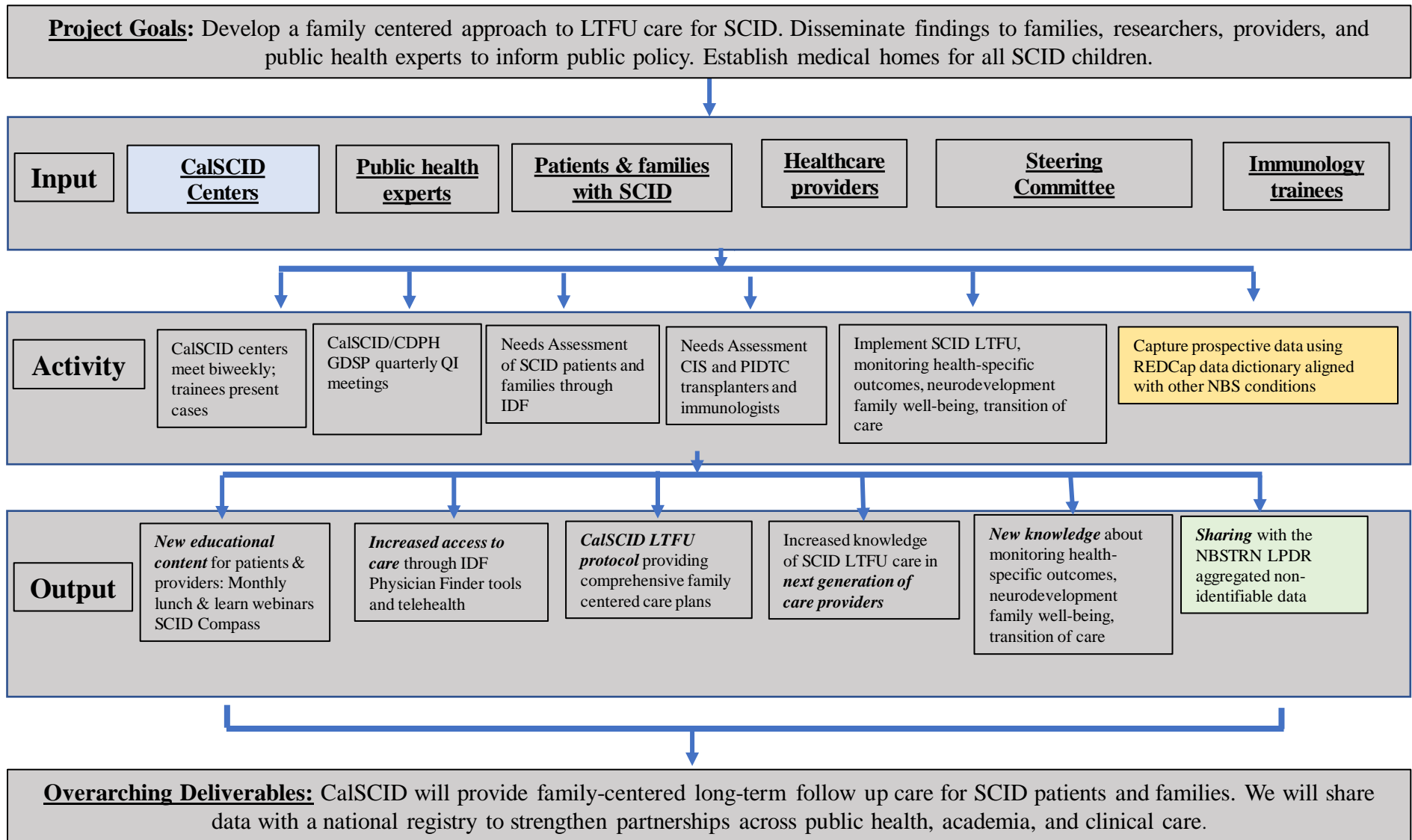


Habits

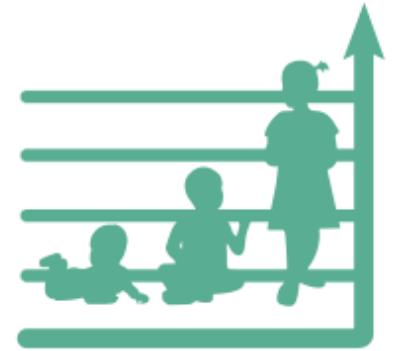
- Exercises regularly
- Date nights with husband
- Follows fitness and foodie influencers
- Regularly posts on Instagram of children
- Online shopping

NEW PATIENT TOOLKIT

 <p>SOCIAL WORK</p>	<p>RESPIRE CARE</p> 
 <p>SCID SUPPORT GROUP</p>	<p>EXERCISE FACILITY</p> 
 <p>MENTAL HEALTH ACCESS</p>	<p>FAMILY EDUCATION</p> 
 <p>MARRIAGE COUNSELING</p>	<p>REMOTE WORK AREA</p> 
 <p>SPIRITUAL SUPPORT</p>	<p>GENETIC COUNSELING</p> 
 <p>LACTATION CONSULT</p>	<p>EARLY DISCHARGE PLANNING</p> 



Data sharing through LPDR



Longitudinal Pediatric Data
Resource translates new
discoveries into clinical practice.

Offers: Suite of information
technology tools to support
newborn screening researchers.

Requires: Prospective collection,
aggregation and sharing of health
information

National data



Research



Public health

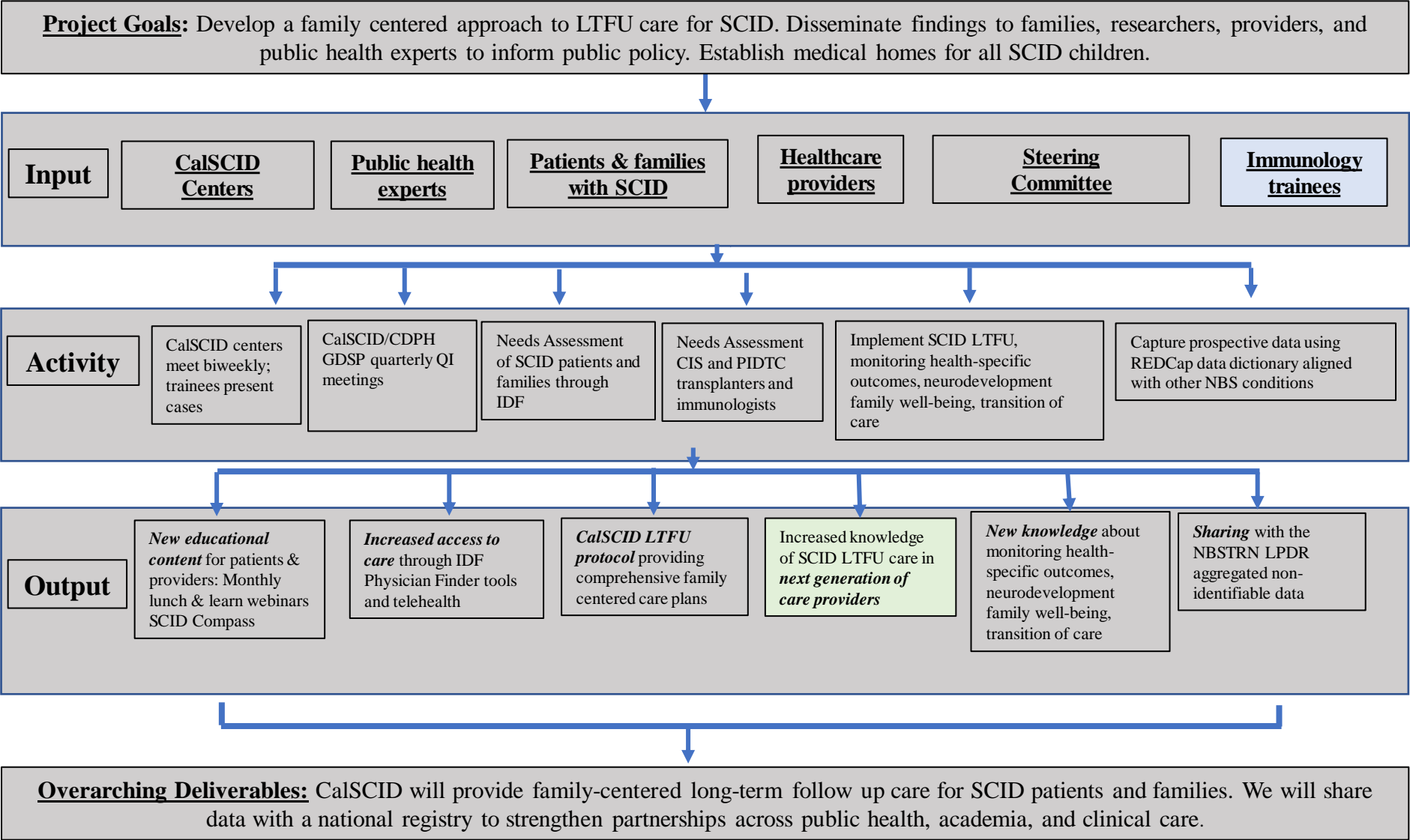


Healthcare quality

Development



Education





Educate the Next Generation of Immunologists



- Current shortage of knowledgeable providers
- We need providers who are familiar with SCID
- Any individual with SCID should be able to identify a medical home

Conclusions

- Provide SCID-specific care throughout the lifespan of patients
- Inform future research
- Influence public policy
- We want to take better care of our patients



Patients and Families



Linda Franck

Manish Butte

Heather Smith

Jennifer Puck

Victoria

Amy Brower



Mort Cowan

Dimitriades

Alissa Creamer



Xin-Hua Chen

David

Emma Mertens

Kathy Nguyen

Buchbinder

Stan Sciortino



Emily Stekol

Cathy Collins

Kelsey Ige

Ami Shah

