

**Important Request to Patients and Families with SCID:  
The Primary Immune Deficiency Treatment Consortium Study of Long-Term Follow-up of  
Severe Combined Immune Deficiency**

The Primary Immune Deficiency Treatment Consortium (PIDTC), a part of the NIH-funded Rare Disease Clinical Research Network (RDCRN), is committed to improving the outcomes for children diagnosed with primary immunodeficiency diseases. A large part of our effort is dedicated to Severe Combined Immune Deficiency (SCID). One of our most critical research efforts, called PIDTC #6902, "A Retrospective and Cross-Sectional Analysis of Patients Treated for SCID," studies long-term outcomes in patients who received a Bone Marrow Transplant (BMT) or who have received enzyme replacement therapy (ERT) for adenosine deaminase (ADA) deficiency and/or gene therapy (GT) for SCID since 1968.

It is extremely important for the PIDTC to learn about individuals with SCID who have been treated over the years to help understand the long-term effects of their underlying genetic conditions as well as side effects of treatments they received. This information will be critical in offering better advice and management for all patients with SCID.

Unfortunately, some individuals with SCID have been lost to follow-up for a variety of reasons. Many live far from the hospital where they received a transplant, and some are adults who "graduated" from pediatric care or felt (or were told) that they don't need to be seen anymore since they were doing well. In addition, some physicians who cared for children with SCID after their transplants have moved away or retired without anyone taking their place.

The PIDTC is very eager to connect with **any person with SCID** who has not been seen at a PIDTC center in the last two years or has not already enrolled in the #6902 Cross-Sectional Analysis study. We wish to enroll all eligible individuals in this long-term follow-up study to get the best picture of how they are doing. This visit would involve the expected evaluation of any patient with SCID and should be covered by insurance or other 3<sup>rd</sup> party payers. The specific research studies will be covered by the PIDTC.

If you have been seen within the last two years at a PIDTC center (see attached list), then you may already have signed up to be in the long-term follow-up study. However, if you were seen and were not told about the #6902 study or don't remember whether you signed a consent form to participate, you can find out about your status and be considered for enrollment at this time. If you have had SCID or are a parent of a child with SCID and are interested in being contacted by the PIDTC to see if you or your child are eligible, please go to the following link: <https://www.rarediseasesnetwork.org/registry/>. Type "SCID" under "How to Join" and complete the information requested.

Once you sign up, the PIDTC will contact you to determine if you are eligible to be part of this study and let you know what PIDTC center is closest to you. In addition to considering enrollment in our #6902 study, we can provide advice on where to receive follow-up care.

If you have any questions, please contact Liz Dunn: [Elizabeth.dunn@ucsf.edu](mailto:Elizabeth.dunn@ucsf.edu)

Thank you,



Mort Cowan, M.D.  
Principle Investigator, PIDTC  
UCSF Benioff Children's Hospital  
San Francisco, CA