

## The APBD Patient Registry

The APBD Patient Registry is critical to developing a treatment and cure for APBD and testing those treatments in clinical trials.

In its simplest form, a patient registry is a list of people who have a particular disease or clinical profile. A simple patient registry will contain names, addresses, and contact information.

In a more complex form, a patient registry can also collect demographic data, family history, and detailed clinical information about each participant's disease experience at multiple points in time.

Over time, especially when 50-100 patients participate and consistently update their records, the clinical data that's collected becomes a natural history study of the disease. This is a tremendous resource for researchers. First, if researchers have a drug that could be used to treat a certain disease symptom, they can determine how many patients have the symptom and if research is practical for that symptom. Secondly, when a study is in the proposal stage, researchers want to be sure that the people who might want to participate in a trial could be identified and informed quickly.

As a detailed registry grows even larger - to 100 or 200 patients, certain commonalities become evident. Doctors or researchers who see just a handful of patients would not necessarily recognize those commonalities.

The APBD Research Foundation and the Mailman School of Public Health at Columbia University, along with a team of international researchers and clinicians, established the APBD patient registry in May 2014. The team at Columbia follows Institutional Review Board (IRB) protocols to ensure the security and privacy of all registrant information.

Registrants always have the option to enroll anonymously, with their physician serving as the point of contact. If they prefer, patients can choose to limit the data they provide. If they prefer, they can even withdraw from participation in the patient registry.