

The A-BOM: Collaborating to identify patient-centered biomarkers and outcome measures for Angelman syndrome. Authors: Terry Jo Bichell, Barbara O'Brien, Anna Arata, Paula Evans, Dan Harvey, Allyson Berent, Tim Bousum, Jodi Cook.

As therapeutic development for Angelman syndrome began to move from bench to bedside, advocacy groups, corporations, clinicians and scientists sought to collaborate by forming a pre-competitive alliance, the Angelman Biomarkers and Outcome Measures Alliance (A-BOM) to identify sensitive, specific and patient-centered BOMs and pilot them. The A-BOM is led by Dr. Terry Jo Bichell, under a steering committee composed of representatives from the Angelman Syndrome Foundation, the Foundation for Angelman Syndrome Therapeutics and Agilis Biotherapeutics.

Immediately after launching, the A-BOM identified crucial areas of focus for Angelman syndrome biomarkers and outcome measures during small meetings between expert researchers and members of the A-BOM steering committee. Focus groups were organized for each area, and priority projects were developed from the input of these groups, resulting in three funded studies, with many more in the development pipeline. One of the first projects to get underway is a Disease Concept Study, funded in partnership with Hoffman-LaRoche, which will serve to inform future outcome measure trials. Through a website and social media, families are informed of the importance of natural history and BOM studies, and are encouraged to participate, an effort which also paves the way for future interactions with government agencies. The A-BOM Alliance is succeeding as a multi-stakeholder, pre-competitive collaboration, sharing data and expertise, which allows for the most efficient and meaningful route to identifying biomarkers and outcome measures which will contribute to the success of future clinical trials.

The pre-competitive model of the A-BOM Alliance can serve as a model for other rare disease groups with multiple family foundations and impending clinical trials. All members are committed to working together to identify the best BOMs for Angelman syndrome. For more information, go to www.angelmanbiomarkers.org.