

SYNGAP 1: PATIENT & CAREGIVER VOICES

Externally Led – Patient Focused Drug Development Meeting

Hosted by



DATE: November 19, 2020 TIME: 12:30 pm – 5:00 pm, EST, U.S. & Canada

TIME	PRESENTATION TITLE	SPEAKERS
12:30 – 12:40 PM	Welcome and Opening Remarks	Monica Weldon, BS; MLS NWU Law Std President/CEO, Bridge the Gap - SYNGAP Education and Research Foundation (BTG)
12:40 – 12:50 PM	Welcoming Remarks FDA Representative	Michelle Campbell, PhD; Senior Clinical Analyst for Stakeholder Engagement, Division of Neurology I, Center for Drug Evaluation and Research, Food and Drug Administration
12:50 – 1:00 PM	Clinical Overview of SYNGAP1	Jimmy Holder, MD, PhD Baylor College of Medicine, Texas Children's Hospital, Co-Chair EL-PFDD Committee, (BTG) Science Advisory Board Member Chair
1:00 – 1:10 PM	Overview of Discussion Format	Moderator: James Valentine, JD, MHS Hyman, Phelps & McNamara, P.C.
1:10 – 1:20 PM	Demographic Polling Questions	SYNGAP1 Family Feedback
1:20 – 1:45 PM	TOPIC #1 PANEL Living with SYNGAP1: Disease Symptoms and Daily Impact	Moderator: James Valentine, JD, MHS Hyman, Phelps & McNamara, P.C. Panelists: Jenny F. – Mother of Child with SYNGAP1 Sharon T. - Mother of Child with SYNGAP1 Christi B. - Mother of Child with SYNGAP1 Valli S. - Mother of Child with SYNGAP1 Hana W. – Person with SYNGAP1
1:45 – 2:45 PM	TOPIC #1 LARGE GROUP DISCUSSION & POLLING Moderator: James Valentine, JD, MHS, Hyman, Phelps & McNamara, P.C. Discussion Leaders: Eric V., Julie M., Maria V., Micky., & Frederic D.	
	<ol style="list-style-type: none"> Of all the symptoms of SYNGAP1, which 1- 3 symptoms have the most significant impact on you or your child's life? <ol style="list-style-type: none"> Which symptoms most affect you or your child now? Which symptoms were the most significant at other times in you or your child's life? What were the first symptoms that you detected (where you suspected something was off, or when looking back after diagnosis you now believe to be SYNGAP1) <ol style="list-style-type: none"> Which symptoms were the most significant at other times in you or your child's life? What were the first symptoms that you detected (where you suspected something was off, or when looking back after diagnosis you now believe to be SYNGAP1) How does SYNGAP1 affect you or your child on best and on worst days? Describe your best days and your worst days? Are there specific activities that are important to you or your child that you or your child cannot do at all or as fully as you would like because of SYNGAP1? <ol style="list-style-type: none"> How does this affect relationships/friendships with others? 	

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	<p>b) How does it affect life activities (school/work, abilities, relationships, self-sufficiency, living situation, activities, etc.)? c) If you or your child could do one activity that you or your child currently are unable to, what would it be?</p> <p>4. How have you or your child’s symptoms changed over time? How has the ability to cope with the symptoms changed over time?</p> <p>5. What do you fear the most as you get older for either yourself or for your child? What worries you most about your condition? What capabilities are most concerned about potentially losing as you grow older? What frustrates you most about your condition?</p>
2:45 – 3:00 PM BREAK	
3:00 – 3:10 PM	<p style="text-align: center;">Challenges of SYNGAP1 Clinical Trial Design</p> <p style="text-align: right;">Constance Smith-Hicks, MD, PhD Kennedy Krieger Institute Co-Chair EL-PFDD Committee (BTG) Science Advisory Board Member</p>
3:10 – 3:35 PM	<p style="text-align: center;">TOPIC #2 PANEL</p> <p style="text-align: center;">Perspective on Current and Future Approaches to Treatment</p> <p style="text-align: right;">Moderator: James Valentine, JD, MHS Hyman, Phelps & McNamara, P.C.</p> <p style="text-align: right;">Panelists: Kevin R. - Father of Child with SYNGAP1 Amit M. - Father of Child with SYNGAP1 Marta D. - Mother of Child with SYNGAP1 Sandra A. - Mother of Child with SYNGAP1 Chantel B. - Mother of Child with SYNGAP1</p>
3:35 – 4:35 PM	<p style="text-align: center;">TOPIC #2 LARGE GROUP DISCUSSION & POLLING</p> <p>Moderator: James Valentine, JD, MHS, Hyman, Phelps & McNamara, P.C. Discussion Leaders: Solenne L., Maarja K-L., Brenda W., Karen A., & Jaime R.</p> <ol style="list-style-type: none"> What are you currently doing to manage your SYNGAP1 symptoms or your child’s symptoms? <ol style="list-style-type: none"> Which specific SYNGAP1 symptoms do the treatments address? How has this treatment regime changed over time and why? How well do these treatments treat the most significant symptoms of SYNGAP1? <ol style="list-style-type: none"> How well do these treatments improve the ability to do specific activities that are important in daily life? What are the most significant downsides to you or your child’s current treatments and how do they affect daily life? (Examples of downsides may include bothersome side effects, going to the hospital for treatment, etc.) Short of a complete cure, what specific things would you look for in an ideal treatment for SYNGAP1? When considering a new treatment, what factors are important to you? <ol style="list-style-type: none"> What questions/data/information would you want to consider participating in a trial? Imagining that there were multiple treatment options approved, what factors into your decision?
4:35 – 4:45 PM	<p style="text-align: center;">Summary Remarks</p> <p style="text-align: right;">Larry Bauer, Senior Regulatory Drug Expert, Hyman, Phelps & McNamara, P.C.</p>
4:45 – 4:50 PM	<p style="text-align: center;">Closing Remarks & Next Steps</p> <p style="text-align: right;">Monica Weldon, President/CEO (BTG)</p>

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