**SYNGAP 1: PATIENT & CAREGIVER VOICES**  
Externally Led – Patient Focused Drug Development Meeting  

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

<table>
<thead>
<tr>
<th>TIME</th>
<th>PRESENTATION TITLE</th>
<th>SPEAKERS</th>
</tr>
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<tr>
<td>12:30 – 12:40 PM</td>
<td>Welcome and Opening Remarks</td>
<td>Monica Weldon, BS; MLS NWU Law Std President/CEO, Bridge the Gap - SYNGAP Education and Research Foundation (BTG)</td>
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<td>12:40 – 12:50 PM</td>
<td>Welcoming Remarks FDA Representative</td>
<td>Michelle Campbell, PhD; Senior Clinical Analyst for Stakeholder Engagement, Division of Neurology 1, Center for Drug Evaluation and Research, Food and Drug Administration</td>
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| 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.  
1. Of all the symptoms of SYNGAP1, which 1–3 symptoms have the most significant impact on you or your child’s life?  
a) Which symptoms most affect you or your child now?  
b) Which symptoms were the most significant at other times in you or your child’s life?  
c) 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)  
b) Which symptoms were the most significant at other times in you or your child’s life?  
c) 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)  
2. How does SYNGAP1 affect you or your child on best and on worst days? Describe your best days and your worst days?  
3. 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?  
a) How does this affect relationships/friendships with others? |
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2:45 – 3:00 PM  BREAK

3:00 – 3:10 PM
Challenges of SYNGAP1 Clinical Trial Design
Constance Smith-Hicks, MD, PhD
Kennedy Krieger Institute
Co-Chair EL-PFDD Committee
(BTG) Science Advisory Board Member

3:10 – 3:35 PM
TOPIC #2 PANEL
Perspective on Current and Future Approaches to Treatment
Moderator: James Valentine, JD, MHS
Hyman, Phelps & McNamara, P.C.
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

3:35 – 4:35 PM
TOPIC #2 LARGE GROUP DISCUSSION & POLLING
Moderator: James Valentine, JD, MHS, Hyman, Phelps & McNamara, P.C.
Discussion Leaders: Solenne L., Maarja K-L., Brenda W., Karen A., & Jaime R.

1. What are you currently doing to manage your SYNGAP1 symptoms or your child’s symptoms?
   a) Which specific SYNGAP1 symptoms do the treatments address?
   b) How has this treatment regime changed over time and why?

2. How well do these treatments treat the most significant symptoms of SYNGAP1?
   a) How well do these treatments improve the ability to do specific activities that are important in daily life?

3. 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.)

4. Short of a complete cure, what specific things would you look for in an ideal treatment for SYNGAP1?

5. When considering a new treatment, what factors are important to you?
   a. What questions/data/information would you want to consider participating in a trial?
   b. Imagining that there were multiple treatment options approved, what factors into your decision?

4:35 – 4:45 PM
Summary Remarks
Larry Bauer, Senior Regulatory Drug Expert,
Hyman, Phelps & McNamaara, P.C.

4:45 – 4:50 PM
Closing Remarks & Next Steps
Monica Weldon, President/CEO (BTG)