Bronchopulmonary Dysplasia (BPD) - FDA-Requested Listening Session

March 20, 2023

Objectives of Session

To gain a better understanding of the experience of individuals who have or had Bronchopulmonary Dysplasia (BPD); the experience of living with or caring for a loved one with BPD; the impact of symptoms on family, caregivers, and activities of daily living and how this impact has changed over time; what they would consider a meaningful improvement in those symptoms; treatment preferences; and their experience with clinical trials.

Discussions in FDA Listening Sessions are informal and not meant to replace, but rather complement, existing patient engagement opportunities in the Agency. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report summarizes the input provided by persons from the BPD community at the meeting. To the extent possible, the terms used in this summary describe the health needs, perspectives, preferences and impacts reflect those of the individual participants. This report is not meant to be representative of the views and experiences of the entire BPD population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.

Summary of Discussion by Question

Round 1: Experience and Needs

1. **What has it been like to live with BPD or care for your child with BPD? Please include how your experience with BPD changed with your or your child's age/development?**
    - One caregiver indicated that their child spent extended time in NICU before going home.
    - One parent shared that their child had to be medically paralyzed to ensure the ventilator was working to its fullest abilities.
    - An adult patient noted they were born at 27-weeks and spent the first six weeks of life intubated.
    - All participants said that they or their children came home from the hospital on oxygen.
    - Both parents stressed how fearful they are for their children's health during the COVID-19 pandemic.
    - The adult patient described their symptoms as leveling off when they were younger before increasing in severity after they turned 30.
    - The adult patient shared that their lung function maintains at 50% and drops to 30% when sick. They suffer from asthma exacerbations and have been intubated several times.

    a. **Follow-up question: Can you share more background about your child and experience as a preemie?**
        - One parent shared that their child was born at the beginning of the COVID-19 pandemic and spent 90 days in NICU. Their child came home on oxygen at 3-months-old and was off oxygen at 6-months-old.

2. **What healthcare-related products are your most important need?**
    - One parent said that they need a product that helps the oxygen apparatus stay in place while their child is using it such as a product that peels and sticks to the skin.

- Another parent shared that their child is high-acuity and requires many healthcare products. Oxygen was noted as the biggest need. As a toddler, their child is active and runs through oxygen tanks quickly. The caregiver also mentioned cloth tracheostomy pads that they buy through Etsy.
- The adult patient noted that they've found a medication cocktail has worked the best for their health needs. They have experienced increased symptoms as they get older. Medical products mentioned included: Trilogy ventilator (described as the most vital product), Dupixent®, and oxygen.

a. Follow-up question: Can you elaborate on the additional need?

- A caregiver provided a list of all the healthcare products they have delivered to meet their child's needs. The products included: saline bullets, Q-tips used to clean stoma, a G-tube stoma, oxygen tubing monthly, syringes, a bubbler for oxygen concentration, and circuits for ventilators. The parent shared that their child is on BiPAP every night. They also said that they use a humidifier. Nebulizer supplies come once every three months.

3. Follow-up question: Have you experienced challenges with insurance coverage for other medications that are not specifically approved for pediatric patients?

- One caregiver stated they haven't had issues with insurance coverage for their healthcare product needs.
- Another caregiver shared that they take Symbicort® for their own exercise-induced asthma but insurance doesn't cover it. Their child used Stelara®, but Medicaid would not cover it as it is not approved for children by the FDA. The caregiver's child also qualifies for home nursing, but they have run into hurdles with hours being taken away.
- The patient participant also expressed hurdles obtaining healthcare products. A previous insurance provider covered everything including multiple intensive care stays. More recent insurance providers have led to difficulties obtaining medications, and as a result, the participant has had to change medications twice.

Round 2: Symptoms and Treatments

4. Of all the symptoms that you/your child experiences because of BPD, can you tell us which 1-2 symptoms has the greatest impact on your/your child's life? Please describe how these symptoms specifically have impacted you/your child and if/how this impact has changed over time.

- One of the parents said that the BPD symptom that has the greatest impact on their life and their child's life is fear of further lung damage and an overabundance of caution when it comes to preventing the spread of illnesses.
- Another parent shared that their child's need for oxygen has had the greatest impact on the family's life. Their child also has issues with CO_2 retention during the night. The need for oxygen is impacting their child's development as they are tripping over tubing as they learn to walk. The need for oxygen also requires the use of a ventilator making it more difficult to travel.
- The adult patient participant noted that functionality has had the biggest effect on their life. Breathing exacerbations have worsened over time leading to frequent hospital visits. This has impacted their ability to lead a normal professional life often having to work from a hospital

bed.

5. **Follow-up question: Apart from respiratory symptoms, are there other symptoms that have a large impact on your and/or your child's life?**
   - One parent shared that their child also suffers from hearing loss.
   - Both parents said that their children suffer from vision issues, including retinopathy of prematurity, possibly due to hypoxia in the NICU. Both parent participants also shared that their children are slightly behind other children of similar age in terms of development.
   - The adult patient said that they have had two head injuries as a result of a lack of oxygen to the brain. The first head injury caused cognitive problems including short-term memory loss and a stutter. They also lost their vision partially and are now legally blind.

6. **If you could create a treatment (i.e., drug or medication) for BPD, what would be the first and most important symptom you would want to improve or treat?**
   - One caregiver stressed they would like to see an improvement in the time to recover after illnesses for their child. They noted that it can take their child with BPD weeks to recover from a cold.
   - Another parent to a child with BPD stated that they would like if there was a way to grow lung tissue more quickly. Their child is growth hormone deficient, and the growth hormone treatment they have received hasn't spurred lung tissue growth. In order for their child to be weaned off of oxygen, they first need to grow healthy lung tissue.
   - The adult patient shared that they would like to be able to function. They are close to incorporating a second biologic into their healthcare regimen, and the hope is that it along with Dupixent® helps reduce breathing exacerbations. If a second biologic leads to more success, it will allow the patient to move away from the need to take steroids.

   a. **Would you consider taking/giving your child a potential drug/treatment that decreased the severity of the symptom, rather than completely removing or resolving it? Why or why not?**
      - All three participants answered yes. They absolutely would take/give their child a potential drug/treatment that decreased the severity of the symptom, even if it didn't remove it or resolve it entirely.
      - Both caregivers stressed the importance of finding a drug/treatment to help make their children's lives a bit easier.
      - The adult patient said that they often tell their medical team they are willing to try anything that will allow them to continue parenting their children.

   b. **If a potential treatment didn't provide relief quickly (e.g., within a couple of days to a week), how long would you be willing to continue taking/giving your child the drug before you/your child started to feel relief from your symptoms from BPD?**
      - Both caregivers said they would be willing to wait however long their child's physician said they should.
      - One parent said they had a conversation about the potentially life-threatening risks of Synagis® when their child first came home from the hospital, but they believed the benefits outweighed the risks.
      - The adult patient shared that a willingness to wait on any relief would be dependent on the context. They stated that if their doctor said a drug/treatment was their only option

they would take it.

9. **Follow-up question: Looking at your child's care that is needed, if there is one thing that could disappear or made better, what would it be?**
    - The parent caregiver stated that their child's need for oxygen would be the symptom they would most like to disappear. Their child often becomes hypoxic after too much activity which can lead to trouble breathing, lethargy, and a change in their child's lip color. .

10. **Follow-up question: Following up on the need for oxygen as one of your biggest challenges, if there were a new drug being evaluated during the NICU stay, what would be an important outcome to show that the drug worked? (E.g., Ability to go home without oxygen, going home sooner)**
    - One parent said that an important outcome for a successful drug would be if their child left the hospital after being born without needing oxygen.
    - An adult patient and a caregiver both stressed the importance of strengthened or preserved lung function as an important outcome for any new drug.

11. **Follow up-question: If there was a treatment in the NICU that allowed your child to get off O_2 earlier, how much risk would you accept?**
    - Both parents explained that it would depend on how certain they were of the potential risk/reward. Both shared that if they were told their child had a 25% chance of survival or a 25% chance a treatment would work, they would take the treatment. However, if doctors were uncertain of the odds/long-term impact they would say no.
    - The adult patient said there have been times when they've been severely ill leading to multiple intubations in a short time period. They shared that if a treatment meant an oxygen tube, they would be willing to do that if it meant more time with their children. If a potential treatment came with high risk, they wouldn't do it unless there were no other options.

12. **Follow-up question: Can you comment on your willingness to try a treatment or drug that allows your child to go home without oxygen, but may not change how your child is doing in the long term, such as after a year?**

    - One parent expressed concern about any treatment being a temporary fix. They'd like something that works in the long run.
    - Another parent shared that they would only do a treatment if they knew whether or not it would be required yearly or if it would help their child's lung improve over time without oxygen.
    - The adult patient said the BPD is unpredictable. They often don't know what the immediate future will look like because symptoms can change quickly. They said that they don't mind using oxygen, so they'd rather stay on oxygen than deal with any potential medication side-effects.

13. **Follow up question: What if the medication is only needed in the NICU, so you can go home without oxygen, but we don't know the long-term effects?**
    - All three participants shared that if a medication was only needed in the NICU they would try it if it meant they or their child could come home without oxygen.

14. Is there anything else you would like to share with FDA regarding your disease/condition?

- They shared that their child is primarily fed via G-tube due to a long period of intubation which prevented them from learning how to chew.
- They also stressed the importance of respite care especially in cases where families do not qualify for Medicare or Medicaid.

FDA Offices & Divisions in Attendance

- **Office of the Commissioner (OC) – *5 offices***
  - OC/OCPP/PAS – Office of Clinical Policy and Programs/Patient Affairs Staff *(organizer)*
  - OC/OCPP – Office of Clinical Policy and Programs
  - OC/OCPP/OCP – Office of Clinical Policy and Programs/Office of Combination Products
  - OC/OCPP/OOPD – Office of Clinical Policy and Programs/Office of Orphan Products Development
  - OC/OCPP/OPT – Office of Clinical Policy and Programs/Office of Pediatric Therapeutics *(requestor)*

- **Center for Biologics Evaluation and Research (CBER) – *5 offices/division***
  - CBER/OCBQ/DIS/PSB – Office of Compliance and Biologics Quality/Division of Inspections and Surveillance/Program Surveillance Branch
  - CBER/OCD/PS – Office of the Center Director/Policy Staff
  - CBER/OTP/OCE/DCEGM/GMB1 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 1
  - CBER/OTP/OCE/DCEGM/GMB2 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 2
  - CBER/OTP/OCE/DCEGM/GMB3 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 3

- **Center for Devices and Radiological Health (CDRH) – *5 offices/divisions***
  - CDRH/OM/DAS/CONT – Office of Management/Division of Acquisition Services/Das Contractors
  - CDRH/OPEQ/OHTI/DHTIC – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IC
  - CDRH/OPEQ/OHTIII -- Office of Product Evaluation and Quality/Office of Health Technology III
  - CDRH/OPEQ/OHTIII/DHTIIIA -- Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III A
  - CDRH/OPEQ/OHTIII/DHTIIIB – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III B

- **Center for Drug Evaluation and Research (CDER) – *11 offices/divisions***

  - CDER/OCD – Office of the Center Director
  - CDER/OCD/PFDD – Office of the Center Director/Patient-Focused Drug Development
  - CDER/OND/ODES/DCOA – Office of New Drugs/Office of Drug Evaluation Science/Division of Clinical Outcome Assessment
  - CDER/OND/OII – Office of New Drugs/Office of Immunology and Inflammation
  - CDER/OND/OII/DHN - Office of New Drugs/Office of Immunology and Inflammation/Division of Hepatology and Nutrition
  - CDER/OND/OII/DPACC - Office of New Drugs/Office of Immunology and Inflammation/Division of Pulmonology, Allergy and Critical Care *(requestor)*
  - CDER/OND/ORDPURM/DRDMG – Office of New Drugs/Office of Rare Diseases, Pediatrics, Urology and Reproductive Medicine/Division of Rare Diseases and Medical Genetics
  - CDER/OTS/OB/DBII – Office of Translational Sciences/Office of Biostatistics/Division of Biometrics II
  - CDER/OTS/OB/DBIII – Office of Translational Sciences/Office of Biostatistics/Division of Biometrics III

Non-FDA Attendees

- Reagan-Udall Foundation for the FDA
- National Organization for Rare Disorders

Participants Represented

- Three participants participated in the session
- One patient over the age of 35 participated
- Two caregivers of children with BPD with ages ranging 2-4 years old

Prior to the Listening Session, Participants Shared

- All patients were premature born at less than 32 weeks of pregnancy.
- The adult patient has participated in a clinical trial previously.

Financial Interest

Participants did not identify financial interests relevant to this meeting and are not receiving compensation for participation in this listening session.