There are NO FDA-approved treatments for Alport syndrome!
Mission is to improve lives through education, empowerment, advocacy, and research

Vision is to prevent kidney failure and hearing loss
Externally-led Patient-focused Drug Development Meeting on Alport Syndrome

August 3, 2018 - Hyattsville, MD
What is an EL–PFDD?

Patients are the experts in what it is like to live with their condition

Primary goal to better incorporate the patient’s voice in drug development and evaluation using:

• Systematic approach
• Best practices for clinical trials
• Patient preferences on benefit and risk outcomes
• Identify information most important to patients to best support their decision making

Outcomes:

• Webcast Recording
• Meeting Transcript
• Summary Meeting Report – Voice of the Patient

Alport Syndrome EL–PFDD

Heard directly from patients and caregivers on key topics:

(1) Patient experience of living with Alport syndrome - disease symptoms and daily impacts
(2) Patient perspective on the current challenges of treatment
(3) Clinical trials
69 attended in person/49 Alport syndrome
98 attended via live webcast

1. I am:
   - An individual with Alport syndrome: 57%
   - A caregiver of someone with Alport syndrome: 43%

2. Where do you live?
   - East coast (Eastern time zone): 42%
   - Mid-west (Central time zone): 31%
   - West (Mountain time zone): 10%
   - West coast (Pacific time zone): 12%
   - Canada: 6%
   - Mexico, Caribbean Islands: 6%
   - Outside of North America (Europe, South America, etc.): 6%

Note: Data from Draft Voice of the Patient document in production and not for public distribution
Demographic Questions

3. What is your age?

4. Do you identify as:

5. What is the length of your illness?

6. Did you receive a diagnosis of:

7. I am:

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Topic 1 (Panel 1):
Living with Alport syndrome
Disease Symptoms and Daily Impacts

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?

2. Are there specific activities that are important to you but that you cannot do at all, or as fully as you would like, because of your condition?
   a) How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?

3. How has your condition and its symptoms changed over time?

4. What worries you most about your condition?
Topic 1: Living with Alport syndrome

Disease Symptoms and Daily Impacts

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Difficulties Experienced

- Hearing loss: 14%
- Anxiety &/or depression: 27%
- Being tired, exhausted, or fatigued: 23%
- Vision problems related to AS: 22%
- Gout: 12%
- Gastrointestinal problems: 12%
- Recurrent infections: 12%
- Swelling (ankles, face, etc.): 12%
- Other: 7%
- I do not have symptoms: 7%

Interference w/Daily Life

- Not at all or minimally: 30%
- Moderately: 42%
- Significant amount: 28%

3 Most Impactful Symptoms

- Hearing loss: 25%
- Anxiety &/or depression: 20%
- Being tired, exhausted, or fatigued: 20%
- Vision problems related to AS: 16%
- Gout: 11%
- Gastrointestinal problems: 11%
- Recurrent infections: 2%
- Swelling (ankles, face, etc.): 2%
- Other: 2%
- I do not have symptoms: 2%

Coping Experiences

- Depression: 17%
- Anxiety: 27%
- Low self-esteem: 10%
- Social isolation: 18%
- Difficulty with relationships outside of family: 11%
- Hopelessness: 15%
- None of the above: 4%

Living with AS

- I miss work or school more than I'm comfortable with: 16%
- Family stress is common in my life: 19%
- Others don't know what it's like live with AS: 27%
- I cannot participate in sports or other physical activities I enjoy: 16%
- My general daily function is limited by AS: 16%
- None of the above: 3%
Topic 2 (Panel 2):
Current Challenges to Treating Alport syndrome

1. What are you currently doing to help treat your condition or its symptoms?
   a) How has your treatment regimen changed over time, and why?

2. How well does your current treatment regimen treat the most significant symptoms of your disease?
   a) How well do your treatments address specific symptoms?
   b) Which symptoms are not addressed as well?

3. What are the most significant downsides to your current treatments and how do they affect your daily life?

4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
Topic 2: Current Challenges to Treating Alport syndrome

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**Medications/Devices**

<table>
<thead>
<tr>
<th>Drug/Device</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>ACE, ARB, bete blockers (water pill)</td>
<td>30%</td>
</tr>
<tr>
<td>Angiotensin (for gout or high uric acid)</td>
<td>4%</td>
</tr>
<tr>
<td>Statin (or other drug for cholesterol)</td>
<td>4%</td>
</tr>
<tr>
<td>Vettassa (or other drug for high potassium)</td>
<td>2%</td>
</tr>
<tr>
<td>Sevelamer (or other drug for high phosphate)</td>
<td>2%</td>
</tr>
<tr>
<td>Anti-depressant or anti-anxiety drug</td>
<td>2%</td>
</tr>
<tr>
<td>Hearing aids</td>
<td>10%</td>
</tr>
<tr>
<td>Glasses (for AS-related vision problems)</td>
<td>10%</td>
</tr>
<tr>
<td>I do not take medication</td>
<td>2%</td>
</tr>
<tr>
<td>I do not use hearing aids or glasses</td>
<td>10%</td>
</tr>
</tbody>
</table>

**ESRD Status**

<table>
<thead>
<tr>
<th>Status/Event</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>I was on dialysis</td>
<td>14%</td>
</tr>
<tr>
<td>I received a kidney transplant</td>
<td>23%</td>
</tr>
<tr>
<td>I have not experienced ESRD</td>
<td>63%</td>
</tr>
<tr>
<td>My patient died while in ESRD</td>
<td>14%</td>
</tr>
</tbody>
</table>

**Mgmt of Symptoms**

<table>
<thead>
<tr>
<th>Symptom Description</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>Very well</td>
<td>10%</td>
</tr>
<tr>
<td>Moderately well</td>
<td>65%</td>
</tr>
<tr>
<td>Poorly or not at all</td>
<td>22%</td>
</tr>
<tr>
<td>I do not currently take any treatments/uses hearing aids or glasses</td>
<td>4%</td>
</tr>
</tbody>
</table>

**Symptoms not Addressed**

<table>
<thead>
<tr>
<th>Symptom Description</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hearing loss</td>
<td>8%</td>
</tr>
<tr>
<td>Anxiety &amp;/or depression</td>
<td>14%</td>
</tr>
<tr>
<td>Being tired, exhausted, or fatigued</td>
<td>14%</td>
</tr>
<tr>
<td>Vision problems related to AS</td>
<td>3%</td>
</tr>
<tr>
<td>Gout</td>
<td>3%</td>
</tr>
<tr>
<td>Gastrointestinal problems</td>
<td>7%</td>
</tr>
<tr>
<td>Recurrent infections</td>
<td>3%</td>
</tr>
<tr>
<td>Swelling (ankles, face, etc.)</td>
<td>5%</td>
</tr>
<tr>
<td>Reduced kidney function (GFR)</td>
<td>12%</td>
</tr>
<tr>
<td>Proteinuria (protein in urine)</td>
<td>17%</td>
</tr>
<tr>
<td>Hematuria (blood in urine)</td>
<td>13%</td>
</tr>
<tr>
<td>Other</td>
<td>3%</td>
</tr>
<tr>
<td>I do not have symptoms</td>
<td>1%</td>
</tr>
</tbody>
</table>

**Likelihood to take drug**

<table>
<thead>
<tr>
<th>Likelihood Type</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>I would not consider taking it</td>
<td>22%</td>
</tr>
<tr>
<td>Not sure</td>
<td>22%</td>
</tr>
<tr>
<td>I would consider taking it</td>
<td>78%</td>
</tr>
</tbody>
</table>

**3 Most Important Factors**

<table>
<thead>
<tr>
<th>Factor Description</th>
<th>Likelihood</th>
</tr>
</thead>
<tbody>
<tr>
<td>Whether drug is taken by mouth, by injection or muscle</td>
<td>7%</td>
</tr>
<tr>
<td>How often you have to take the drug</td>
<td>4%</td>
</tr>
<tr>
<td>Evidence in AS patients that drug improves specific symptoms most bothersome to you</td>
<td>25%</td>
</tr>
<tr>
<td>Number of side effects known for the drug</td>
<td>6%</td>
</tr>
<tr>
<td>Severity of side effects known for the drug</td>
<td>28%</td>
</tr>
<tr>
<td>Cost and/or whether covered by insurance</td>
<td>16%</td>
</tr>
<tr>
<td>What your physician recommends</td>
<td>13%</td>
</tr>
<tr>
<td>Other</td>
<td>4%</td>
</tr>
</tbody>
</table>
Topic 2: Current Challenges to Treating Alport syndrome

Most Important for Future Therapy

- Evidence that the drug will reverse decline in kidney function (i.e., halt progression of AS, delay need for dialysis), but has no effect on hearing: 74%
- Evidence that the drug significantly improves hearing, but has no effect on kidney function: 2%
- Evidence that the drug will improve my quality of life or prevent future reduction in quality of life: 17%
- Evidence that the drug will prolong my life: 8%

Daily Life Interference

- Symptoms from AS: 45%
- Side effects from medicines you take for AS: 7%
- Both: symptoms and side effects are equal: 24%
- I can't tell the difference between effects of AS and side effects from medicines: 14%
- I do not have symptoms or side effects from medicines: 10%

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Topic 3: Clinical Trials Questions

1. What is your experience in, and perception of, clinical trials for a new kidney disease drug?

2. Would you enroll in a clinical trial if it required:
   a. No kidney biopsy
   b. 1 kidney biopsy within 1 year
   c. 2 kidney biopsies within 1 year
   d. 3 kidney biopsies within 1 year

3. Of the following factors related to a test drug in a clinical trial, select up to 5 that rank as most important to your decision to participate:
   a. Placebo
   b. Stop current treatment
   c. Potential side effects
   d. How drug administered
   e. Effectiveness for benefits most meaningful
   f. Commitment to participate
   g. Frequency of appointments
   h. Distance to trial site
   i. Length of trial
   j. Kidney biopsy required
   k. Negative things heard about trials
   l. Other
Topic 3: Clinical Trials

5 Most Important Factors to Participate

Experience and Perception

Kidney Biopsy Requirement

Note: Data from Draft Voice of the Patient document in production and not for public distribution
Key Themes
Summary of Key Themes

- Fatigue
- Pregnancy Complications
- Family Planning
- Hearing Loss
- Gout, GI Issues, Repeated Infections
- Willingness to Participate in Trials
- Side Effects and Eligibility Criteria