Cystinosis Patient Survey
Data Analysis Final Report

August 30, 2010

Reaching healthcare influencers, producing measurable results.
Scope of Research

1. Partnership initiated with CRN to reach patients and families with cystinosis.

2. JPA fielded survey via online, mail and phone calls – July 17 through present

3. Total responses to date: 192
   • US = 162
   • Ex-US = 21
   • Unknown = 9

* The following analysis is based on US surveys
Key Takeaways

• While the vast majority (94%) of cystinosis patients have experienced crystal formation, only 61% currently use the eye drops.

• For those that aren’t using the drops, it is primarily because they haven’t been recommended or the patient doesn’t experience symptoms; Currently, price is not a major factor in use of the drops.

• The primary source of the eye drops is NIH Eye Clinic.

• Most patients start using the drops over 1 year after cystinosis diagnosis. Compliance in terms of daily use and storage varies.

• The majority (89%) of patients have health insurance, but household income varies widely among patients and families.
### Cystinosis Diagnosis

<table>
<thead>
<tr>
<th>Age</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>3.4 years</td>
<td><strong>Average</strong> age at cystinosis diagnosis</td>
</tr>
<tr>
<td>Prenatal</td>
<td><strong>Minimum</strong> age at cystinosis diagnosis</td>
</tr>
<tr>
<td>25 years</td>
<td><strong>Maximum</strong> age at cystinosis diagnosis</td>
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</table>
Has the patient experienced cystine crystal formation in his/her eyes?

- Yes: 94%
- No: 4%
- No answer: 2%

Age when crystals were first identifiable:
- Average age = 4.5 years
- Min age = .25 years
- Max age = 28 years

Only 16 respondents indicated that they know their crystal score:
- Min score = 1
- Max score = 8
Eye Drop Usage

Does the patient currently use cysteamine eye drops?

- Yes: 61%
- No: 37%
- No answer: 2%

Cystinosis Patient Survey Preliminary Data Analysis
Why isn't the patient using cysteamine eye drops?

- **Other**: 35%
- **The cost of the drops is too high**: 13%
- **Don't know where or how to get the eye drops**: 13%
- **Uncomfortable side effects**: 12%
- **They expire too quickly/before they can be used**: 3%
- **Challenges due to temperature requirements**: 12%
- **Doctor has not recommended them/does not recommend them**: 33%
- **No current symptoms related to the eyes**: 28%
- **Didn't result in improvement/prevention of crystals**: 3%

Other includes quality of life issues:
- “Stink”
- “Need to use too often”
- “Uncomfortable”
- “Hassle”
- “Symptoms are minimal”

Respondents selected multiple responses.
How long after diagnosis did the patient begin using eye drops?

- **At diagnosis**: 15%
- **Within 3 months of diagnosis**: 7%
- **Between 3 - 6 months after diagnosis**: 5%
- **Between 6 - 9 months after diagnosis**: 4%
- **Between 9 months and 1 year after diagnosis**: 4%
- **Over 1 year after diagnosis**: 66%

Cystinosis Patient Survey Preliminary Data Analysis
Who first prescribed the use of cysteamine eye drops?

- **Other**: 49%
- **Nephrologist**: 9%
- **Ophthalmologist**: 41%
- **Pediatrician**: 0%
- **General practitioner**: 2%

Other includes specific locations and doctors:
- NIH
- Dr. Gahl
- Dr. Kaiser
- Dr. Matalon
Other includes:
• Mayo Clinic
• S&R Drug Company in Kirbyville, TX

Where does the patient receive their eye drops?

- NIH Eye Clinic: 73%
- Compounding pharmacy: 25%
- Directly from doctor/clinic: 2%
- Major/chain pharmacy: 0%
- Other: 6%

Respondents selected multiple responses.
How many times a day does the patient use the eye drops?

- One when the cystine crystals become unbearable: 8%
- Once a day: 0%
- 2-3 times a day: 30%
- Every 2-3 waking hours: 36%
- Every waking hour: 27%
How are the eye drops stored throughout the day?

- Mostly in the refrigerator, but not always: 17%
- At room temperature: 29%
- In a freezer: 8%
- In a refrigerator: 46%
Has the patient experienced a decrease in cystine crystal formation or prevention since using the drops?

- Yes: 88%
- No: 12%
Is the patient likely to use cysteamine eye drops if they become commercially available and accessible at the pharmacy?

- Yes, they are an essential part of treatment: 56%
- Yes, as long as insurance will cover them: 24%
- Yes, as long as the cost is not prohibitive: 17%
- No, the patient does not currently use them anyway: 6%
- Maybe: 12%
Kidney Transplants

Has the patient received a kidney transplant?

- Yes: 50%
- No: 48%
- No answer: 2%

Age at transplant
Average age = 14
Min age = 5
Max age = 56
Health Insurance Status

Does the patient currently have health insurance?

- Yes: 89%
- No: 4%
- No answer: 7%

What type of insurance does the patient have?

- Medicaid: 31%
- Medicare: 19%
- Private through employer: 36%
- Private through parent: 12%
- Other: 0%

Other primarily includes state-run health programs, e.g., Mass Safety Net, Illinois Public Aid
Demographic Information

Total Household Income

- $0 - $25K: 14%
- $25 - $50K: 18%
- $50 - $75K: 20%
- $75 - $100K: 17%
- Over $100K: 19%
- No answer: 12%

Cystinosis Patient Survey Preliminary Data Analysis
Patient Location (n=162)
Cystinosis Information & Resources

Where does the patient/family get cystinosis information and resources?

- Patient advocacy groups: 80%
- Primary care doctor: 18%
- Specialists: 45%
- Nephrologists: 64%
- Internet searchers: 47%
- Nurses: 7%
- Pharma companies: 6%
- Other families/patients: 4%

Respondents selected multiple responses.
Cystinosis Patient Survey Preliminary Data Analysis

Which resources were most helpful at diagnosis?

- Study results: 19%
- Questions to ask doctor/specialist: 29%
- Tips for finding doctor/specialist: 20%
- Chat rooms: 9%
- Support groups: 41%
- Opps to meet other families/patients: 56%
- Financial resources and info: 26%
- Info on the disease: 75%
- Info on treatment options: 56%

Respondents selected multiple responses.
Which resources would be helpful if they were made available?

- Financial help to access treatment: 54%
- Online patient and family support groups: 50%
- In-person meetings with other patients/families: 51%
- Resources, such as brochures and fact sheets: 51%
- Info re doctors experienced in treating cystinosis: 74%
- Other: Trial/study information: 10%

Respondents selected multiple responses.
Please direct any questions to:

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