Patient Perception of Oxbryta Treatment Benefit

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Disclosures

- Speakers Bureau: Global Blood Therapeutics
- Contracted Research: Global Blood Therapeutics, Novartis, Pfizer
- Advisory Board Membership: Global Blood Therapeutics, Novartis
Study Design and Methods

- The UTH Standing Data Collection Protocol allows collection and publication of anonymized patient data collected for standard clinical care. This was the data primary source.
- The protocol was amended to allow assessment by the Patient Global Impression – Improvement scale (PGI-I) which is not part of the standard data collection protocol.
- Oxbryta was added to existing SCD management regimens, some of which included HU.
- Clinical data was collected on 27 consecutive patients with SCD, all with at least 8 weeks of data collection. Four patients lacked complete laboratory and PGI-I data.
- Data analysis was done on 23 patients for whom there was information on Hb, retic %, CGI-I, and PGI-I.
  - 17 had complete laboratory data.
  - 23 patients provided PGI-I information.
- Laboratory testing frequency was limited by COVID-19 pandemic restrictions.
Clinical Global Impressions Scale*

- Questionnaire developed and validated originally for use in NIH-sponsored clinical trials. Provides a brief, stand-alone assessment of the clinician's view of the patient's global functioning prior to and after initiating a study medication.

- CGI
  - Correlates well with standard, well-known research drug efficacy scales (30+ yrs. experience).
  - Used in FDA-regulated trials including mental health, incontinence and fibromyalgia.
  - CGI-Severity Scale (CGI-S): assesses patient severity after treatment intervention.
  - CGI- Improvement Scale (CGI-I): assesses patient improvement after treatment intervention.

- Patient Global Impression- Improvement (PGI-I)
  - Questionnaire of patient’s impression of improvement with treatment intervention.

Assessment of Improvement after Treatment by Clinician (CGI-I) and Patient (PGI-I)

1. Very much better
2. Much better
3. A little better
4. No change
5. A little worse
6. Much worse
7. Very much worse

Results

- 23 patients, ages 20 to 66 years
- 65% female
- HbS genotypes: SS, 91%; Sβ0-thalassemia, 9%; Sβ+-thalassemia, 0%; SC, 0%

<table>
<thead>
<tr>
<th>Table 1</th>
<th>Patient Number</th>
<th>Mean Change from Baseline</th>
<th>Standard Deviation</th>
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<tbody>
<tr>
<td>Hb g/dL</td>
<td>17</td>
<td>1.14</td>
<td>1.378</td>
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<tr>
<td>Reticulocytes, %</td>
<td>17</td>
<td>-4.21</td>
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<tr>
<td>Total bilirubin mg/dL</td>
<td>17</td>
<td>-1.014</td>
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</tbody>
</table>
Patient and Clinician Assessment Improvement with Oxbryta Therapy

Results (cont.)
Hemoglobin Change and Clinical Improvement

CGI-I and PGI-1

Patient Assessment on Both Scales as Either

1 or 2 (Greatly improved or Improved)

3 or 4 (A little improved or no change)
Results (cont.)

Figure 3.
Typical Peripheral Blood Smear
Before and After X Weeks of Oxbryta Treatment
About 30% of the patients reported mild to moderate diarrhea. The issue was managed by reduction in Oxbryta dose until diarrhea improved. Patients then were titrated back to the recommended dose. For more severe diarrhea, Oxbryta was interrupted for 2 to 3 days followed by restart at a lower dose, with or without the addition of anti-diarrheal medications.

All patients were alerted to the possibility of diarrhea at Oxbryta initiation. They were advised to not stop treatment without contacting the physician for guidance on management.
Conclusions

- This is the first study assessment of patient improvement with Oxbryta using the 7-point PGI-I and CGI-I scales
- Most patients were very much improved or much improved
  - PGI-I: 16 of 23 (70%)  
  - CGI-I: 19 of 23 (83%)
- A minority of patients were a little improved or not changed
  - PGI-I: 7 of 23 (30%)  
  - CGI-I: 4 of 23 (17%)
- Some patients noted significant improvement in energy and less pain at their typical sites of chronic pain while on Oxbryta therapy
- Clinical improvement occurred for some patients whose Hbs declined or improved only slightly from baseline
- This study suggests that most sickle cell disease patients perceive benefit with Oxbryta therapy in terms of improvement in their condition and level of functioning