The Impact of COVID-19 on the Rare Disease Community

Perspectives from NORD and IQVIA

 Alone we are rare. Together we are strong.

August 4, 2020
This webinar is being recorded.
Submit your questions using the chat function. It can be found at the **left hand side** of the window.
Upcoming Webinar

Screen Baby Screen: Perspectives on Newborn Screening
August 27, 2020 at 3:00pm EDT

Jose Abdenur, MD
Chief, Division of Metabolic Disorders
Children’s Hospital of Orange County

Danyelle Sun
Rare Mom
Wisconsin State Ambassador
NORD Rare Action Network

Register:  https://bit.ly/2Pi4VMX
Speaker Introductions

Pamela Gavin
Chief Strategy Officer
NORD

Vanessa Boulanger, MPH
Director of Research
NORD

Jeffrey Keefer, MD
VP, Medical Strategy
Head of Pediatric
and Rare Disease Center
of Excellence
IQVIA

Ali Smyth, PhD
Strategy Director
Pediatric and Rare Disease
Center of Excellence
IQVIA

Murray Aitken
SVP & Executive
Director Institute for
Human Data Science
IQVIA

rarediseases.org
Opening Remarks
Impact on Patient Community

Presented by Vanessa Boulanger
NORD’s COVID Response: Surveying the Community

Goal: Understand the Experience of Patients with Rare Diseases Dealing with the Impact of COVID

Activities: NORD Surveyed the community twice. Once in April and then again in June

Respondents: A total of 1,600+ responses were collected. 36% of participants contributed to both surveys.

Outcome: Our analyses have been published on NORD’s website, shared by the FDA, and cited in a statement sent to the World Health Organization by international patient advocacy organizations
Respondents by Age and Category

Capacity At Which Questions Were Answered

- Patients: Apr-20 68%, Jun-20 71%
- Caregivers: Apr-20 22%, Jun-20 22%
- Family members: Apr-20 25%, Jun-20 5%
- Other: Apr-20 1%, Jun-20 1%

Age of Individuals With Rare Disease

- Pediatric (0-17): Apr-20 17%, Jun-20 14%
- Adult (18+): Apr-20 83%, Jun-20 86%
Respondents by Disease Area

Disease Areas

- Autoimmune/Autoinflammatory diseases: 24%
- Bacterial infections: 21%
- Behavioral/mental disorders: 18%
- Blood diseases: 18%
- Chromosome disorders: 13%
- Congenital and genetic diseases: 13%
- Connective tissue diseases: 13%
- Digestive diseases: 11%
- Ear, nose, and throat diseases: 11%
- Endocrine diseases: 11%
- Environmental diseases: 11%
- Eye diseases: 11%
- Female reproductive diseases: 11%
- Fungal infections: 11%
- Heart diseases: 11%
- Hereditary cancer syndromes: 10%
- Immune system diseases: 6%
- Kidney and urinary diseases: 6%
- Lung diseases: 5%
- Male reproductive diseases: 5%
- Metabolic disorders: 5%
- Mouth diseases: 5%
- Musculoskeletal diseases: 5%
- Nervous system diseases: 5%
- Nutritional diseases: 5%
- Parasitic diseases: 5%
- Rare cancers: 5%
- Skin diseases: 5%
- Viral infections: 5%
- Undiagnosed: 5%
- Other: 0.70%
- NA: 0.20%
- Other: 2%
- Other: 2%
- Other: 2%
- Other: 1%
- Other: 1%
- Other: 0.10%
- Other: 0.02%
General Concerns and Fears

How worried are you about COVID-19?

<table>
<thead>
<tr>
<th></th>
<th>Apr-20</th>
<th>Jun-20</th>
</tr>
</thead>
<tbody>
<tr>
<td>Not Worried</td>
<td>2%</td>
<td>6%</td>
</tr>
<tr>
<td>Somewhat Worried</td>
<td>31%</td>
<td>36%</td>
</tr>
<tr>
<td>Very Worried</td>
<td>37%</td>
<td>37%</td>
</tr>
<tr>
<td>Extremely Worried</td>
<td>30%</td>
<td>21%</td>
</tr>
</tbody>
</table>

How much has COVID-19 impacted you and your family?

<table>
<thead>
<tr>
<th></th>
<th>Apr-20</th>
<th>Jun-20</th>
</tr>
</thead>
<tbody>
<tr>
<td>Not Impacted</td>
<td>5%</td>
<td>7%</td>
</tr>
<tr>
<td>Somewhat Impacted</td>
<td>36%</td>
<td>37%</td>
</tr>
<tr>
<td>Very Impacted</td>
<td>39%</td>
<td>38%</td>
</tr>
<tr>
<td>Extremely Impacted</td>
<td>20%</td>
<td>17%</td>
</tr>
</tbody>
</table>
Common Issues and Concerns

• Not being able to access PPE (masks & gloves)
• Access to testing and risk of false negatives
• Access to medical services
• Financial stability
• Equity of care
• Returning to work with high risk individual at home
• Staying safe now and when this is “over”
• Social isolation
Income and Employment

Has your household been impacted by a loss of income due to COVID-19?

Has anyone in your household lost their job, temporarily or permanently, due to the coronavirus (COVID-19)?

Loss of Health Insurance As a Result of Income or Employment Changes

11% in April and 9% in June
Have you had a medical appointment canceled due to COVID-19?

- Yes: Apr-20 74%, Jun-20 79%
- No: Apr-20 20%, Jun-20 16%
- NA: Apr-20 7%, Jun-20 5%

Have you experienced challenges accessing medical care or treatment due to COVID-19?

- Yes: Apr-20 38%, Jun-20 59%
- No: Apr-20 32%, Jun-20 16%
- NA: Apr-20 8%, Jun-20 8%

Access to Medical Care
Access to Telehealth

April 2020
- Offered a telehealth alternative: 59%
- Not offered a telehealth alternative: 32%
- NA: 9%

June 2020
- Offered a telehealth alternative: 83%
- Not offered a telehealth alternative: 13%
- NA: 4%
Access to Telehealth

88% of those offered a telemedicine appointment accepted it

92% of those who had a telemedicine appointment said it was a positive experience

70% would like the option of telemedicine appointments for every appointment in the future

61% said that the primary reason that they would opt out of a telemedicine visit would be because they prefer face-to-face interaction

June 2020
In June, **46%** reported that they needed to access PPE prior to COVID-19 to help manage infection risks related to their rare disease (18% constantly; 28% sometimes)
Impact on Rare Disease Patient Organizations
Impact to Organizations

Key fundraising events cancelled = significant loss in income (avg. of 30-60%)

- For larger organizations (> $1M) this equates to more than $750k in loss revenue, and in some cases more than $3 million
- For smaller organizations (< $1M) this equates to between $15,000 - $100,000
- Impacts their ability to fund research grants, research centers, in addition to patient care and services

Cutting Expenses

- Many expenses for organizations are “fixed” leaving little room to cut superfluous costs
- Outcome - Reducing staff, canceling office leases, cutting programs (including research)

If revenue projections do not change, some smaller organizations may need to close temporarily or permanently
Impact on rare disease clinical research
IQVIA’s portfolio in rare disease covers diverse disease areas

Rare disease studies ongoing at IQVIA with COVID-19 response strategies approved by the sponsor
Rare disease study continuity during the COVID-19 pandemic

Rare disease studies ongoing at IQVIA with COVID-19 response strategies approved by the sponsor

In development
Start up
Enrolling
Enrollment complete, study ongoing

1. No change needed
2. Change in study, study continues
3. Some study activities on hold
4. All study activities on hold
COVID-19 Clinical Trial Continuity

Solutions that have been deployed that allow study continuity
Study continuity – rare respiratory study

*Patients first approach enabled rapid response to ensure study continuity*

**Challenge**

- Highly complex and challenging study with many assessments that needed to be conducted at a physical site
- Due to COVID-19, patients cannot travel to sites and sites cannot perform all assessments

**Response**

1. **Protocol simplification** – Reassessed the protocol and removed study activities that were not critical
2. **Patient diaries** – Moved to electronic patient reported outcomes (eCOA) for electronic diary entries
3. **Drug delivery and lab assessments** – Implemented drug delivery and home-health services for at-home administration and lab draws
4. **Remote visits** – Pushed to telehealth visits to ensure continuity of contact between site and patient

**Results**

Rapid response ensured study continuity

**Challenge**

- Due to COVID-19, patients cannot travel to sites and sites cannot perform all assessments

**Response**

1. **Office-based patient assessments removed** – 1 week to convert to electronic diaries
2. **Home visits** – 47 home visits completed 1 month post outbreak, 67 to date
Continued recruitment - rare inborn error metabolism

2 patients identified including cross-border referral during global lockdown

**Challenge**
- Potentially live saving treatments should still be administered to patients during the COVID pandemic
- For rare disease patients these are often experimental

**Response**
- Obtained hospital and government approvals
- Ensured site had staff and resources to conduct study
- Organized transportation
- Organized rental accommodation, facilitated cleaning

**Results**
1. One patient referred into study site from another part of Spain, dosed at height of lockdown
2. One cross-border referral currently planned

Study stayed on track for recruitment during global pandemic
Rare disease patient visits have declined each month during the pandemic, but are showing signs of recovery regionally.

- Source: IQVIA Trial Management Data Hub (CTMS, EDC) - Baseline Pre-Covid: November 2019
The change in US rare disease patient visits observed was heterogeneous across states.

Source: IQVIA Trial Management Data Hub (CTMS, EDC) - Baseline Pre-Covid: November 2019
Site willingness to conduct CRA visits remotely has grown since the start of lockdown

Source: IQVIA Trial Management Data Hub (CTMS, EDC); - Baseline Pre-Covid : November 2019
Impact on approved drugs available to patients with rare disorders
Total volume of orphan drugs has been less dynamic than the overall market since the beginning of the pandemic.

Source: IQVIA sales weekly (WSP) July 17, 2020

Note: Orphan-only drug means drugs with single or multiple orphan indications.
Volume through retail channels was less affected than non-retail channels, but the recovery trajectory is less clear.

Orphans-only retail volume vs total retail volume 2020

Orphans-only retail volume was 19% higher than baseline.

Orphans-only non-retail volume vs total non-retail volume 2020

Orphans-only non-retail volume was 26% higher than baseline.

Source: IQVIA sales weekly (WSP) July 17, 2020
Note: Baseline = average of Jan (excluding 3rd Jan) and Feb weeks
Orphan-only drug means drugs with single or multiple orphan indications.
New therapy starts of orphan drugs have declined almost 20% since the end of February

Source: IQVIA New to Brand weekly, July 17, 2020
The Outlook for Rare Diseases
Questions?