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1 EXECUTIVE SUMMARY

There are almost 7,000 rare diseases, which affect millions of patients. Currently, there are treatments for only about 5% of these diseases. Barriers inhibiting the development of treatments and cures include lack of alignment among all stakeholders, lack of an adequate technological infrastructure, lack of adequate data and data interoperability, and dated regulatory and reimbursement frameworks.

The COVID-19 pandemic has shown what is possible when stakeholders are aligned, collaborate with a sense of urgency, and leverage technology to accelerate progress.

The Rare Disease Advisory Committee convened by InformaConnect and OpenText sees a better way. This group envisions an easy-to-use online platform that contains comprehensive longitudinal health data about patients. This data will be accessible by patients, providers, researchers, payers, policy makers, and enabled via technology partners. The platform and data standards will be developed collaboratively by multiple stakeholders and will be used to improve care and to develop new treatments.

Ultimately, this platform will not just be systems of records that house data; it will become systems of insights used throughout the healthcare ecosystem to help develop treatments and improve the delivery of care—becoming systems of engagement and systems of experiences.

Critical to realizing this vision is trust, collaboration, and alignment among the various stakeholders. Needed are standards for data integration and interoperability, so data can flow seamlessly into and out of the platform. Patients must be engaged in the creation of this platform and must have control over their data as well as assurances regarding privacy and the use of the data. The platform must be easy to use and patient advocacy groups need the ability to leverage the platform to help create rich registries for their diseases.

Turning this vision from a concept into a tangible reality requires a scalable, connected, technological backbone and infrastructure. This infrastructure must enable and support a host of technologies and processes including big data analytics, low-code business applications, connected IoT devices, and much more. To help create this technological background, stakeholders need to forge relationships with technology partners that are focused on rare diseases, have deep life sciences and healthcare industry knowledge, and have a broad portfolio of disruptive technology solutions.

Call to Action

This is our vision for the future. It is one enabling step toward the ultimate vision of rare disease patients having as much therapeutic choice as patients with more prevalent conditions, for new patients to be able to understand more about their condition(s), and to provide a platform for engagement, understanding, and synergy. We call upon other key stakeholders (such as NORD, FoCR, EURODIS, and other rare leaders) to join us in developing a pilot platform to demonstrate how this can work before scaling across rare diseases.
WHY RARE DISEASES ARE SO IMPORTANT

Rare diseases are not so rare. Per the National Human Genome Research Institute, there are 6,800 rare diseases, each of which affects fewer than 200,000 people in the U.S. at any given time. In total, rare diseases affect an estimated 25 to 30 million Americans.²

Globally, approximately 300 million people, representing 4% of the world's population, have a rare disease.³ (Note: the definition of a “rare disease” differs across countries.)

However, despite the prevalence of rare diseases and the millions of people who are affected, as of 2020, since passage of the Rare Disease Act in 1983, there have only been 599 orphan products approved by the FDA.⁴ This means there are currently no approved treatments for over 90% of all rare diseases.

For patients with rare diseases, and for their families and loved ones, this is an incredibly challenging journey that can go on for years or even decades. Patients are frequently misdiagnosed. They often bounce between doctors and hospitals seeking answers, and frequently try a series of ineffective treatments. It is a physically difficult, emotionally deflating, and often extremely expensive experience.

In fact, a recent study from IQVIA found that the average annual cost of orphan drug therapies is $32,000 and 39% of drugs with orphan indications cost more than $100,000 annually.⁵ In many situations, patients have to bear some of these high costs, which can limit access to these treatments.

It is essential that multiple stakeholders, representing all aspects of the healthcare system, come together to find a better way to develop treatments for those patients afflicted by rare diseases.
RARE DISEASE ADVISORY COMMITTEE

To accelerate the development of cures for rare diseases, Informa Connect and OpenText assembled a Rare Disease Advisory Committee.

Members of the committee came from a range backgrounds, functions, and organizations. They represented pharmaceutical companies and patient advocacy organizations, with experience in science, R&D, patient support services, policy, advocacy, compliance, and more. Many of the individuals on the committee have been personally affected by rare diseases and all have a deep passion for this field.

Rare Disease Advisory Committee

Leader:
Ferdi Steinmann, Sr. Global Industry Strategist, Life Sciences, OpenText

Members:
Thomas Abbott, Head, Real World Evidence, Astellas Pharma US
Paul Edwards, Chief Strategy Officer, Infectious Disease Connect
Peter Hawkins, Sr. Director, Head of Rare Genetic Disease Risk Management, Agios Pharmaceuticals
Jen Horonjeff, Founder & CEO, Savvy Cooperative
Rahul Khara, Vice President, Legal & Chief Compliance Officer, Acceleron Pharma
Tricia Mullins, Global Head, Patient Advocacy, Atara Biotherapeutic
Megan Murphy, Global Lead, Pipeline Patient Advocacy, Biogen
Mark Rutter, Sr. Director, Global Regulatory Policy, Biogen
Jeffrey Smith, Sr. Director, Head, Patient Advocacy, Wave Life Sciences
Tracey Walsh, Head of U.S. Patient Support Services, Sanofi Genzyme
Martine Zimmerman, Global Head of Regulatory Affairs, Alexion Pharma

The Rare Disease Advisory Committee gathered virtually three times in November 2020. The purpose of this Committee and focus of these meetings was:

- Establishing and confirming the current reality for rare diseases and identifying major challenges
- Defining a vision of the desired future state
- Describing barriers to achieving this vision
- Determining key leverage points for proceeding toward the vision
- Identifying opportunities, recommendations, actions, and initiatives
## CURRENT CHALLENGES IN DEVELOPING TREATMENTS AND CURES FOR RARE DISEASES

Among representatives from pharmaceutical companies and other organizations focused on rare diseases, several important challenges were identified. The three primary challenges in developing treatments are **data**, **small numbers of patients to conduct clinical trials**, and **cultural barriers** within the industry.

<table>
<thead>
<tr>
<th>Primary Challenge</th>
<th>Description</th>
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<tr>
<td><strong>Data</strong></td>
<td>Data-related challenges pertaining to rare diseases include:</td>
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<tr>
<td></td>
<td>• The breadth and depth of data required for research and approval are significant. This can include genomic data, EHR data, and claims data, as well as environmental and behavioral data.</td>
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<tr>
<td></td>
<td>• Difficulty accessing data, as it often resides in silos which prevents data from being shared and aggregated.</td>
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<td></td>
<td>• Lack of standards on data and interoperability.</td>
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<td>• Issues related to patient consent, privacy, security, which prevents data sharing. To date, regulations have not adequately dealt with privacy issues and there is regulatory inconsistency between countries.</td>
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<td>• Questions about who owns and can access data, and how it is used.</td>
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<tr>
<td><strong>Small numbers</strong></td>
<td>Because there are so few patients with rare diseases, especially ultra-rare diseases, there are challenges identifying enough patients for clinical studies and for real-world post-approval data gathering. There are also challenges studying diverse populations.</td>
</tr>
<tr>
<td><strong>Culture</strong></td>
<td>Within organizations, a major barrier is inertia and the attitude of, “This is the way we've always done it.” Despite the need for progress, many organizations are averse to change.</td>
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Other challenges include:

- **Engaging patients** early in the process when developing drugs and getting patients access to expensive treatments.
- **Lack of agile regulatory frameworks** that apply to rare diseases, particularly outside of the US and EU, resulting in regulation that slows the process of getting treatments to patients.
- **Dated reimbursement models** that were not developed for small patient populations, set unrealistic evidence requirements for innovators, and do not incentivize the generics and biosimilar leaders to provide appropriately timed competition to off-patent innovators.
- **An unaligned ecosystem without common standards or frameworks.** Stakeholders, who often have their own expectations and incentives, frequently work in their own silos, behaving as adversaries not collaborators.
IMPACT OF THE COVID-19 PANDEMIC

The COVID-19 pandemic is having a profound impact and is transforming multiple aspects of healthcare. Five ways in which healthcare is being transformed are:

1. **A shift in mindset—to emphasize speed and collaboration.** Healthcare has typically proceeded slowly and cautiously, with each organization in its own silo. The pandemic has changed that. In providing PPE for workers and treatments for patients, supply chains have operated faster than ever. And to develop vaccines on incredibly fast timing, companies have partnered and collaborated in unprecedented ways. There has also been a greater spirit of collaboration between industry and government regulators to remove barriers and move quickly. This represents a major cultural shift, which those involved with rare diseases hope to emulate.

2. **Greater empathy for patients.** As patients with COVID have suffered in isolation, without a vaccine or cure, this has heightened the understanding of patients with rare diseases. As one committee member observed, “The vast majority of people who don’t have a rare disease are now living the life of a rare disease patient. This is the way people with rare diseases have lived their whole life. It’s eye opening.”

3. **Increased attention on diversity, equity, and inclusion.** As companies have sprinted into trials of newly developed vaccines, they have faced challenges testing with diverse, representative populations. This illustrates the importance—and difficulty—of ensuring diversity, equity, and inclusion throughout the processes of developing treatments for rare diseases.

4. **Greater use of technology.** One of the most significant changes during the pandemic has been acceleration in the adoption and use of technology, particularly telehealth. While telehealth previously existed, the pandemic has given it a giant push. It has changed how patients interact with physicians and made it easier for patients to access hard-to-reach specialists.

   There has also been accelerated use of wearable technologies which can collect data via the Internet of Things (IoT), and which can be used in concert with telemedicine. Artificial intelligence is the key being used to mine and analyze this data, looking for insights.

5. **A new level of flexibility in research processes.** To keep clinical research moving forward during the pandemic, researchers have been creative and have adapted. There has been increased flexibility in conducting decentralized trials and using remote monitoring. The flexibility during the pandemic gives researchers hope that there can be greater research flexibility post the pandemic.
Pandemic’s Impact on Drug Development

The pandemic created a sense of urgency that forced participants in the healthcare ecosystem to innovate and collaborate to move more quickly. The progress in developing a vaccine, caring for patients, and continuing with research through new types of models has shown what is possible. It is expected that many of these transformations will be sustained and built upon to produce faster ways of operating, which can be leveraged to accelerate development of treatments for rare diseases.

At the same time, the pandemic has exposed continuing gaps in the completeness of data and in the lack of data interoperability. For example, if a patient being treated in one hospital goes across the street to another hospital, their health data may be incomplete and not be available. In a crisis, not having complete data and seamless interoperability hurts the ability to deliver high-quality care.

VISION FOR THE FUTURE

The current moment presents a unique opportunity for those who are passionate about rare diseases to come together to reimagine how to accelerate development of treatments.

A unifying vision for a better future is:

- **An easy-to-use interoperable, online data platform** that contains comprehensive longitudinal data about patients that can be accessed by each of the 6 P’s.

*Figure 1. The 6 P’s of the Life Sciences and Healthcare Ecosystem*
Each of these stakeholders will be more likely to participate in interoperable data platforms when they see benefits

- **Patients**: Gain a high-quality, connected experience across all healthcare interactions and better manage their own healthcare.
- **Providers**: Improve the quality, effectiveness, and timeliness of treatments and become more patient-centric by focusing on better patient outcomes.
- **Policymakers**: Build better partnerships with industry, help improve the quality of regulatory submissions, and accelerate the submissions process.
- **Payers**: Reduce costs and minimize the risk of fraud while helping improve the quality of care provided and new, targeted products being delivered.
- **Product research & manufacturing**: Improves and streamlines the clinical trial process and boosts the quality and efficiency of new product development and manufacture.
- **Partners (Technology & vendors)**: Ensure software applications that integrate, communicate, and exchange PHI efficiently and securely across the entire healthcare ecosystem.

- This platform will be able to be **leveraged by multiple patient advocacy groups to create rich patient registries** for different rare and ultra-rare diseases. By leveraging this platform, each organization and disease registry doesn’t need to reinvent the wheel.
- This **platform and the data standards and rules will be developed collaboratively** by multiple stakeholders—including regulators—with a sense of urgency.
- Ultimately, this technological platform will not just be systems of records that house data; it will **become systems of insights** used throughout the healthcare ecosystem to help develop treatments and improve the delivery of care—becoming systems of engagement and systems of experiences.
This vision for an interoperable data platform provides the following benefits to each of the 6 P’s:

<table>
<thead>
<tr>
<th>6 P’s</th>
<th>Benefits</th>
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<tbody>
<tr>
<td><strong>Patients</strong></td>
<td>• Trust that treatments are based on accurate and timely information</td>
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<tr>
<td></td>
<td>• Can easily and securely share healthcare data with providers, payers, and other stakeholders</td>
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<tr>
<td></td>
<td>• Able to better manage own healthcare via chosen devices and online patient communities</td>
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<tr>
<td><strong>Providers</strong></td>
<td>• A ‘single source of the truth’ for every patient</td>
</tr>
<tr>
<td></td>
<td>• Up-to-date, accurate and compliant information delivered where and when it’s needed</td>
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<tr>
<td></td>
<td>• Frees frontline staff to spend more time with patients and focus on outcomes</td>
</tr>
<tr>
<td><strong>Policy makers</strong></td>
<td>• Improves the quality of data available to drive better decision making</td>
</tr>
<tr>
<td></td>
<td>• Creates collaboration-based partnerships with industry players and other stakeholders</td>
</tr>
<tr>
<td></td>
<td>• Speeds new drug approvals and better monitors post market performance</td>
</tr>
<tr>
<td><strong>Payers</strong></td>
<td>• Gain greater insight into drug and treatment performance</td>
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<tr>
<td></td>
<td>• Provide increasingly targeted treatment solutions that reduce fraud and block poor healthcare interventions</td>
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<tr>
<td></td>
<td>• Develop new policies and plans based around identified patient needs</td>
</tr>
<tr>
<td><strong>Product research &amp; manufacturing</strong></td>
<td>• Improves the recruitment and performance of clinical trials</td>
</tr>
<tr>
<td></td>
<td>• Improves the speed and effectiveness of transitioning from research to production</td>
</tr>
<tr>
<td></td>
<td>• Improves quality and efficacy in the way products and manufactured and delivered</td>
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<tr>
<td><strong>Partners</strong></td>
<td>• Improve the connectivity and interoperability of new and existing products and solutions</td>
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<tr>
<td></td>
<td>• Help bring new products to market faster based on insight from real-world patient data</td>
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<td></td>
<td>• Develop a new generation of patient-specific systems and apps</td>
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</table>
Creating the Data Platform of the Future

Scalable Data Platform

- **System of Records**
  - Data integration
  - Data management
  - Data visibility
  - Analytics
  - Delivered through managed services

- **System of Insights**
  - **Data Type**
    - Structured, Semi- and Un-Structured
    - Paper forms
    - Limited digitized content
  - **Scalability**
    - Collaboration
      - Internal/External

- **System of Engagement**
  - **Data Type**
    - Data from IoMT, wearables, social media, etc.
  - **Personalization**
    - based on each patient’s needs
    - “… right treatment at right time with agility and flexibility”

- **System of Experience**

Today | Evolution of the Data Platform over time | Future

“We’re sort of transforming the health data ecosystem, using rare diseases as a vehicle for doing that.”

— Participant in Rare Disease Advisory Committee
# Critical Pillars of Realizing This Vision

In realizing this vision there are several critical elements – which we term as essential “Pillars” that must be addressed. These pillars include:

<table>
<thead>
<tr>
<th>Pillars</th>
<th>Description of Essential Pillar</th>
</tr>
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<tbody>
<tr>
<td>Environmental</td>
<td>Realizing this vision requires an aligned and integrated ecosystem where stakeholders work together.</td>
</tr>
<tr>
<td>Behavioral &amp; Cultural</td>
<td>There must be trust among all stakeholders based on common goals, agreed upon standards, and a willingness to share data. Creating this platform requires internal collaboration across all functional areas within manufacturers and external collaboration among all stakeholders, as opposed to competition. Collaboration must include pre-competitive sharing of information and broad use of best practices by industry members and stakeholders.</td>
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<tr>
<td>Data</td>
<td>It is essential for data to flow seamlessly into the platform. Because so much of the data in healthcare is unstructured data, it is impossible to have uniform data (unless mandated by governing authorities). Technology must play a critical role in harmonizing and standardizing unstructured and semi-structured allowing for data integration and data interoperability.</td>
</tr>
<tr>
<td>Patient Engagement</td>
<td>Patients must have early and frequent input into the creation of the platform. On an ongoing basis, it isn't the responsibility of patients to populate data into the platform. But patients must be able to consent to have their data included, need the ability to control their data, must be assured of privacy of their data, and must have access to their data. Also, patients want their physicians to be able to access their data and want to be able to collaborate with other physicians. In exchange for consenting to share their data, patients may receive some form of value on an ongoing basis. This value might include awareness to potential trials, receiving updates regarding relevant scientific discoveries, and other continuous activities that promote patient engagement.</td>
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### Convenience & Access

An essential requirement of this common platform is simplicity and **ease of use for patients**, with a user-friendly front end that is analogous to a consumer-facing platform such as Facebook. However, the platform must be a secure portal that provides all relevant stakeholders with access to data based on their role in the healthcare ecosystem.

### Leveraging the Platform for Registries

There must be the ability for organizations such as **patient advocacy groups** to leverage the platform to create **rich registries** for their disease containing comprehensive longitudinal information.

### Roles & Responsibilities

It is essential to have greater private sector **collaboration with regulators through public-private partnerships** along with increased **flexibility by regulators**. One example: for rare diseases with small populations, it is important for regulators to have the flexibility to have the ability to approve a drug based on **modeling** and then gather real-world evidence based on in-market use. This is currently being considered in very limited cases but is not yet broadly used.

### Technological

The entire vision is for a next generation platform, which requires a resilient and flexible modern technology infrastructure with strong data interoperability capabilities.

However, while the technological infrastructure is essential, the technology itself is only a platform and a tool for sharing data to improve the delivery of care. This tool must be used collaboratively within organizations – from R&D through commercialization and across stakeholders.

### Pilot

As described in the introduction of this paper, the task before us is a large one. Through the realization of the above pillars, we propose to pilot this new technology in a rare disease. Criteria for selecting a potential rare disease area to pilot might include:

- The existence of therapeutic options
- An active R&D pipeline
- Access to patient data
- An ecosystem of stakeholders willing to participate in the pilot, such as pharmaceutical companies and patient associations
THE ROLE TRANSFORMATIONAL TECHNOLOGY WILL NEED TO PLAY

Turning this vision from a concept into a tangible reality that provides the potential to accelerate the development of treatments for rare diseases will be a process that takes time and requires the collaboration of all stakeholders. Importantly, realizing this vision requires a scalable, connected, technological backbone and infrastructure. This infrastructure must enable and support a host of different critical technologies including (this is only a partial list):

- Connected IoT devices with multiple sensors that provides a wealth of data
- Omni-Channel Intelligent Capture—OCR/ICR
- Big Data analytics to glean insights from this data. Capabilities must include embedded AI, analytics, and reporting.
- Low-code business application/case/process management
- An integrated data platform with enterprise content management (ECM) solutions
- Compliant information archiving
- Remote access
- Security solutions with identity access management (IAM)

These technologies will need to work seamlessly on premise and in a cloud environment.

Though technology will be instrumental in this transformation, it will nearly be impossible to achieve the vision without domain expertise from life sciences and healthcare as well as information management (IM) capabilities across the entire journey of data, from creating data to decommissioning it.
Key capabilities in the IM journey in life sciences and healthcare are:

- **Capture & Digitize**: In healthcare, manual data entry is becoming a thing of the past. The IM journey starts with intelligent technology, like intelligent OCR, that can capture, digitize, and convert large volumes of information – from sources such as electronic documents or handwritten clinical notes into searchable text. This first step is critical to have data that is usable in the digital world.

- **Store, Manage & Migrate**: Data must be stored in a centrally managed repository, where it can be accessed by multiple applications. Content management solutions will provide the means for this information to be migrated, organized, and stored within any application.

- **Analyze, Report & Predict**: Being able to identify and analyze rare disease data will be essential, especially for predictive modeling and forecasting.

- **Process & Automate**: Optimizing a digital transformation requires moving from manual to automated processes, especially automating workflow processes across multiple systems.

- **Search & Discover**: Conducting effective investigations and making good decisions depends on getting the right information to the right person at the right time. Finding the correct data can reduce work time, helping stakeholders who are focused on rare diseases quickly find answers. Powerful search and discovery capabilities enable stakeholders to locate and retrieve necessary information, no matter where it resides.

- **Integrate & Access**: Stakeholders working in the rare disease area desire seamless connectivity across multiple systems, information, and processes. Integrating providing access (direct and remote) to solutions and data will accelerate the development of life-saving therapies.

- **Transform, View & Communicate**: Effective communication to stakeholders in the rare disease community relies on information being suitable for its audience in format, layout, and appearance. With suitable information, it will empower stakeholders to create, collaborate, and publish content that is customized and targeted for the needs of the rare disease community.

- **Archiving & Retention**: In addition to storing, managing, and migrating content, it is necessary to archive and retain all records. Governance and compliance of regulated or non-regulated data is essential in rare diseases. Having access to relevant information when and where it is needed, differentiates between just managing in the rare disease space and transforming it.
Developing Your IM Capabilities

To develop the requisite IM capabilities, stakeholders in the rare disease ecosystem can go further, faster by forging relationships with technology partners that have, at a minimum:

- **Deep subject matter expertise** in life sciences and healthcare
- **Outstanding trust and integrity**, with an exceptional customer-centric focus
- **Industry-leading business and strategic thinking** prowess
- **Strong professional services** (LSHC) and customer support
- **A broad, deep portfolio of disruptive technology solutions**
- **This ability to be agnostic in terms of data, data sources, and applications**

WHERE WE GO FROM HERE

This Rare Disease Advisory Committee surfaced multiple challenges, articulated a clear and compelling general future vision, and laid out essential pillars in realizing this vision.

Members of the Committee agreed that articulating this general vision for rare diseases was just the beginning. Far more interactions will be needed with a broader array of stakeholders to add more specificity to this vision and to turn it into a reality that makes a meaningful difference in the lives of individuals with rare diseases. The conversations of this Committee are merely the starting point of a larger collaborative process.

Among the important actions and next steps in making progress toward this vision include:

- Identifying who can lead and drive this effort, which potentially might be a nonprofit or an NGO (non-governmental organization).
- Creating a broad coalition of support by inviting more stakeholders to participate in this conversation.
- Identifying a technology partner(s) with subject matter expertise in Life Sciences and a broad solution portfolio with capabilities to integrate into enterprise legacy systems and new transformative innovative technologies.
As shown below, the ecosystem for rare diseases is incredibly complex. At the center it begins with the patients and caregivers, and includes patient advocates. The ecosystem also includes organizations focused on developing and manufacturing therapies such as pharmaceutical and biotech manufacturers, medical device manufacturers, clinical research organizations (CROs), and contract development and manufacturing organizations (CDMOs). In addition, there are also stakeholders whose main aim is to ensure providing and delivering life-saving treatments to patients; they include healthcare providers, pharmacies, logistics provides, and 3PLs. The entire ecosystem is overseen by regulatory authorities.

*Figure 4. The Complex Array of Stakeholders in the Rare Disease Ecosystem*
Due to the complexity of the ecosystem, and the need for collaboration among all parties, it is essential that representatives from all key stakeholder groups participate in the conversation about accelerating cures for rare diseases by leveraging digital technology.

This Rare Disease Advisory Committee agreed that following stakeholder groups should be included in the next round future discussions:

- **Patient representatives**
  - Patients with rare and ultra-rare diseases
  - Caregivers
  - Representatives from patient advocacy organizations

- Representative from throughout **life sciences and biotech manufacturers** including:
  - C-suite
  - Operations
  - IT
  - Line of business (LoB) including R&D, clinical, manufacturing, and commercial (sales and marketing)
  - Subject Matter Experts (example: specific functional areas such as regulation and privacy)

- Representatives from **across the healthcare ecosystem** such as:
  - Regulators and policymakers (FDA & EMA)
  - Providers (hospitals, clinical research, academia)
  - Payers
  - Vendors (CROs, CDMOs, distribution, etc.)
  - Partners (business consulting, technology, and software developers, such as OpenText)

- Representatives from **rare disease organizations** such as (but not limited to):
  - National Organization for Rare Disorders (NORD)
  - European Organization for Rare Diseases (EURORDIS)
  - Global Genes
  - Every Life Foundation
  - FoCR

**Call to Action**

This is our vision for the future. It is one enabling step toward the ultimate vision of rare disease patients having as much therapeutic choice as patients with more prevalent conditions, for new patients to be able to understand more about their condition(s), and to provide a platform for engagement, understanding and synergy. We call upon other key stakeholders (NORD, FoCR, EURODIS and other rare leaders) to join us in developing a pilot platform to demonstrate how this can work before scaling across rare diseases.
Informa Connect and OpenText wish to thank the committee members for participating in this committee, for sharing their perspectives and ideas, and for their deep commitment to overcoming barriers and pursuing treatments and cures for patients with rare diseases.
OpenText Information Management solutions help Life Sciences organizations leverage the full potential of their information assets. We have extensive capabilities and deliver IM to support for all aspects of the product development and data journey – from research and development (R&D) through to commercialization.

At OpenText we take an IM Solution-centric approach. As was discussed in Figure 2 (page 11), with this approach we take customers from information creation to decommissioning, leveraging the portfolio of tools and capabilities shown below.

**Figure 5. OpenText’s Capabilities**

1. **OpenText CEM**
   - Augments and bridges our customer’s current transactional systems to intelligently create, personalize, deliver, monitor and enable insights driven true anonymous and authenticated omnichannel experiences at scale.

2. **OpenText Confidential. ©2021 All Rights Reserved.**
   - Enables the seamless, secure flow of information across an extended business ecosystem of people, systems, and things; simplify inherent B2B complexities and gain insights to drive efficiencies and speed time to revenue.

3. **OpenText’s Capabilities**
   - Provides a single platform for process automation, case management and low-code application development, to automate complex business processes, enable better decision making and improve customer experience.

4. **Digital forensic & information security solutions**
   - Provides Artificial Intelligence / Machine Learning, analytics, text mining, & Big Data processing combined with enterprise-class Business Intelligence. Data Discovery and reporting. Adds value to EIM through insight, powering the Intelligent, Connected Enterprise.

5. **Manage the entire e-discovery lifecycle**
   - Information management through collection to review and production, with an integrated suite of capabilities to lower e-discovery risks and standardize processes while reducing end-to-end costs.

6. **Pharmaceutical, biotech, and medical device companies trust OpenText to help them meet information management challenges by enabling any-to-any integrations, providing a broad solution portfolio, and delivering flexible paths to the cloud.**

**Life Sciences solutions from OpenText**, combined with OpenText’s information management expertise in the Life Sciences industry, help organizations transform and automate their processes related to asset management, information governance, clinical trials, drug development, and real-world evidence. With its extensive end-to-end portfolio of technologies, OpenText delivers Life Science solutions focused on health outcomes and results.
OpenText, The Information Company™, enables organizations to gain insight through market-leading information management solutions, on-premises or in the cloud. All of the top 20 Life Sciences organizations rely on OpenText's Information Management solutions and team of industry experts.

For more information about OpenText (NASDAQ: OTEX, TSX: OTEX) visit: opentext.com

Contact us to learn more about how OpenText for Life Sciences can help you transform your organizational needs and opportunities.

Ferdi Steinmann is a Global Industry Strategist for Life Sciences at OpenText, with over two decades of experience in the healthcare, life sciences, and pharmaceutical industries. He has tremendous insight into the way technology helps companies succeed.

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Twitter: @OpentextFerdi

1Rare Disease by the Numbers, America's Biopharmaceutical Companies, as of 2/8/21. Link
2National Human Genome Research Institute, Rare Diseases FAQ, as of 12/23/20. Link
3Not So Rare: 300 Million People Worldwide Affected by Rare Diseases, AJMC (American Journal of Managed Care), November 7, 2019. Link
4New Study Investigates the Number of Available Orphan Products, Generics and Biosimilars, NORD (National Organization for Rare Diseases), March 25, 2021. Link
5Orphan Drugs in the United States: Rare Disease Innovation and Cost Trends Through 2019, IQVIA, December 2020. Link