Clarivate

Thinking of zebras

Delivering innovative medicines to patients with rare diseases

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A gastroenterologist, confronted with a patient in wrenching abdominal pain of vexing origin, tells her: "This is one of those cases where you need to shrug your shoulders and say 'I don't know.'" A musculoskeletal specialist advises a patient experiencing painful joint subluxations that "It's in your head." A physiotherapist, assessing a patient whose condition was partially diagnosed by an orthopedic surgeon, says dismissively: "This is not a thing."

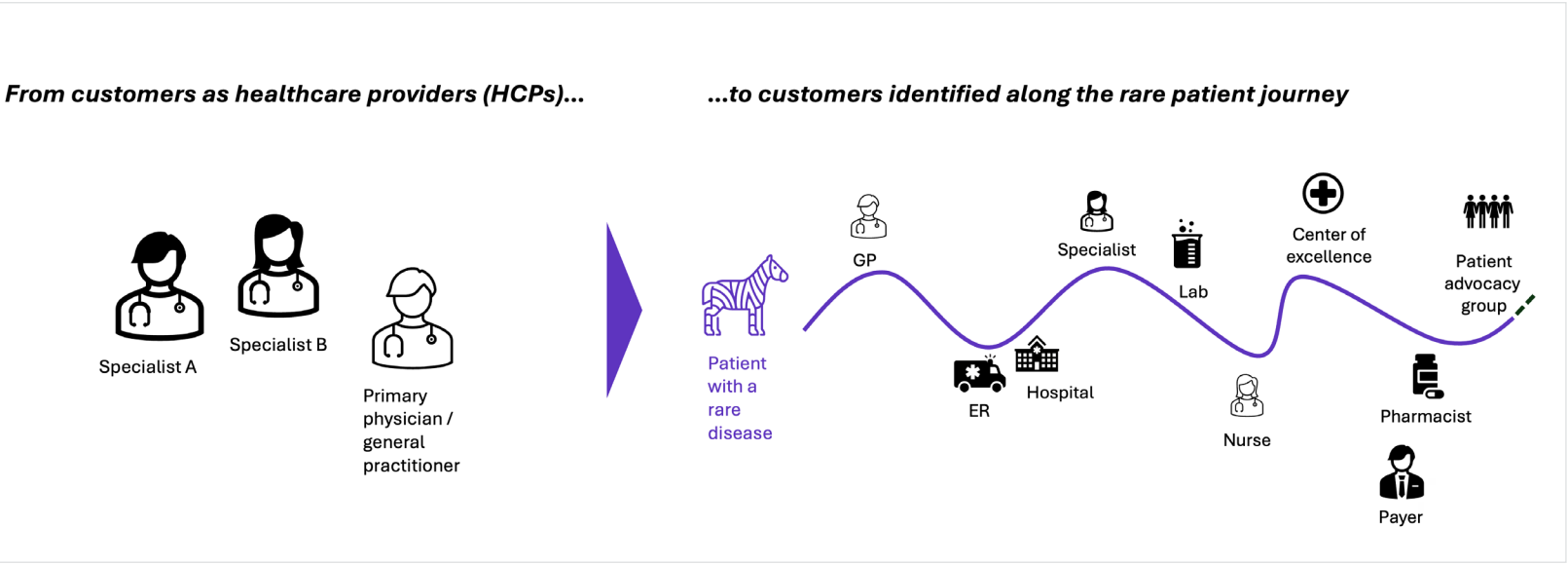
Three stories, one patient — a person with a rare disease whose symptoms and presentations are uncommon and may vary over time. In another setting, this patient might be considered a unicorn. However, in the rare disease space, people in this situation are considered 'Zebras' — a sly play on the foundational medical school diagnostic dictum that when you hear hoofbeats, you should think instead of horses. Each has a unique set of symptoms so uncommon that even the most experienced and best-intentioned physician is unlikely to arrive at a correct diagnosis. Their conditions are rare, often extremely so, but rare diseases, as a whole, are very common, affecting 1 in 12 people in Europe, for example (European Medicines Agency, 2025).

Medical science has identified more than 7,000 of these diseases (NORD, 2025), which affect an estimated 300m people worldwide (Nguengang Wakap et al, 2019), but therapeutic options remain limited. Despite growing public awareness and interest in this space from biopharmaceutical companies, a little over 500 treatments have been approved by the Food and Drug Administration (FDA) and the European Medicines Agency (EMA) to date.

Recognizing zebras along the patient journey

Taking a patient-centered approach to drug development and commercialization is nowhere more critical than when working in the rare disease space. Every patient's condition and circumstances are unique, and patients with a rare disease are typically navigating a bewildering array of providers, institutions and, in some cases, payers in order to manage their complex disease. Moreover, developing treatments for these diseases can be resource intensive, and each patient is essential to the commercial viability of these endeavors.

Figure 1: Taking a patient-first perspective in the rare disease journey



Source: Clarivate Analytics

Patient identification

Anchoring a patient-first approach in the patient journey can help identify potential opportunities for treatments entering or expanding into the space. Rare diseases are defined differently from market to market — as those affecting one in 2,000 people in Europe (EMA, no date) or fewer than 200,000 in the United States (FDA, 2025) — but with very low prevalence rates, every patient counts. This is particularly true in ultra-rare diseases, defined in Europe as affecting fewer than one in 50,000 (OHE, 2024).

On average, it takes these patients five years just to get a diagnosis (EURORDIS, no date). Raising awareness, fostering referral pathways and enhancing screening options are therefore critical. Even after diagnosis, poor awareness of treatment options and reliance on outmoded standard-of-care treatments often prevent patients from accessing more efficacious and well-tolerated therapeutics.

Treatment

To ensure patient access to optimal treatments, companies must facilitate care pathways by supporting providers with up-to-date information on diseases and emerging treatments and engaging multi-disciplinary teams of providers as well as carers. Understanding the patient and carer experience at the treatment stage helps to foster optimal follow-up and better adherence.

Bespoke patient support programs can help support patients with particularly challenging needs — for example, patients with moderate-to-severe disease who are experiencing acute symptoms or pediatric patients transitioning to adult services. UCB's ONWARD™ initiative is a good example of this, providing personalized support via care coordinators as well as treatment support and symptom tracking tools to patients receiving ZILBRYSQ® (zilucoplan) or RYSTIGGO® (rozanolixizumab) for generalized myasthenia gravis (UCB, no date).

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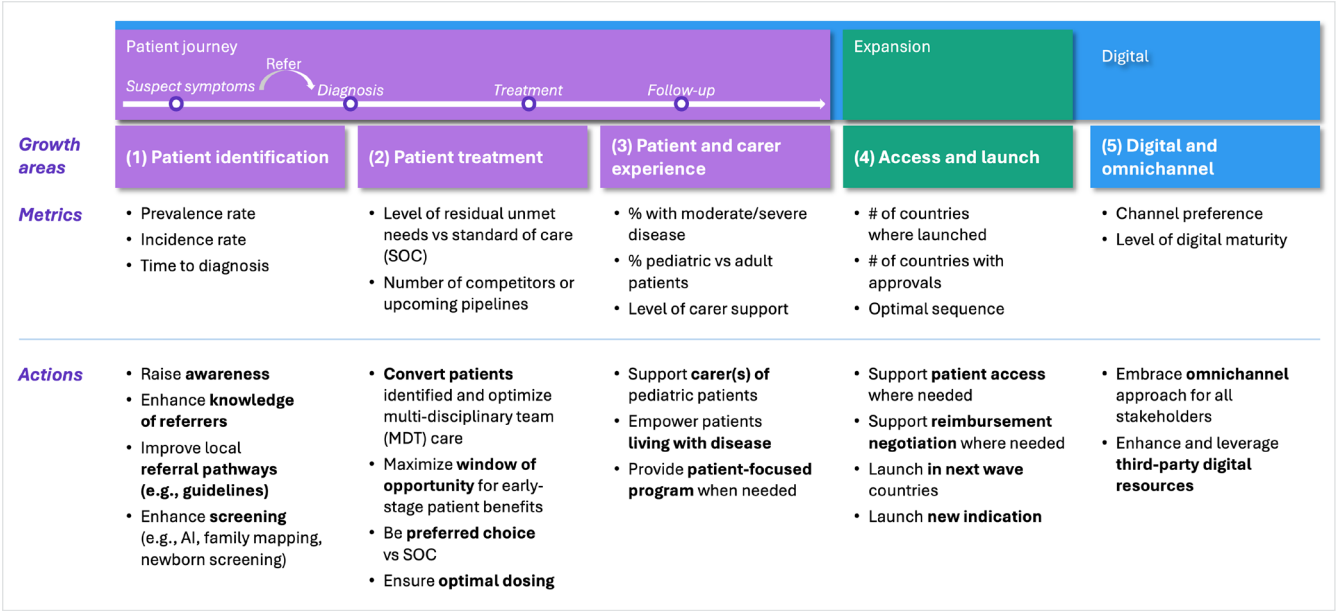
Access and launch

Optimizing commercialization in the rare disease space requires taking a cross-functional approach to the patient journey, starting with access and launch of new medicines and continuing through product launches and expansion into new indications and geographies. Both the U.S. FDA and the EMA have established paths to market for orphan drugs, providing specific incentives and patent protections for innovators. Within the European market, this will evolve further with the upcoming Joint Clinical Assessment rollout for new orphan drugs from 2028 (as it has for Advanced Therapy Medicinal Products this year), designed to standardize clinical assessment across the European Union.

Embracing digital-first and omnichannel approaches has allowed innovators to reach rare disease patients, carers and other stakeholders at scale, allowing for varied channel preferences and digital maturity levels. Digital media are also revolutionizing research and development — for example, Alexion has developed digital biomarkers to enhance their understanding of patients for their clinical programs and to reach patients in underserved geographies.

Figure 2: Understanding and actioning the rare disease patient journey



Source: Clarivate Analytics

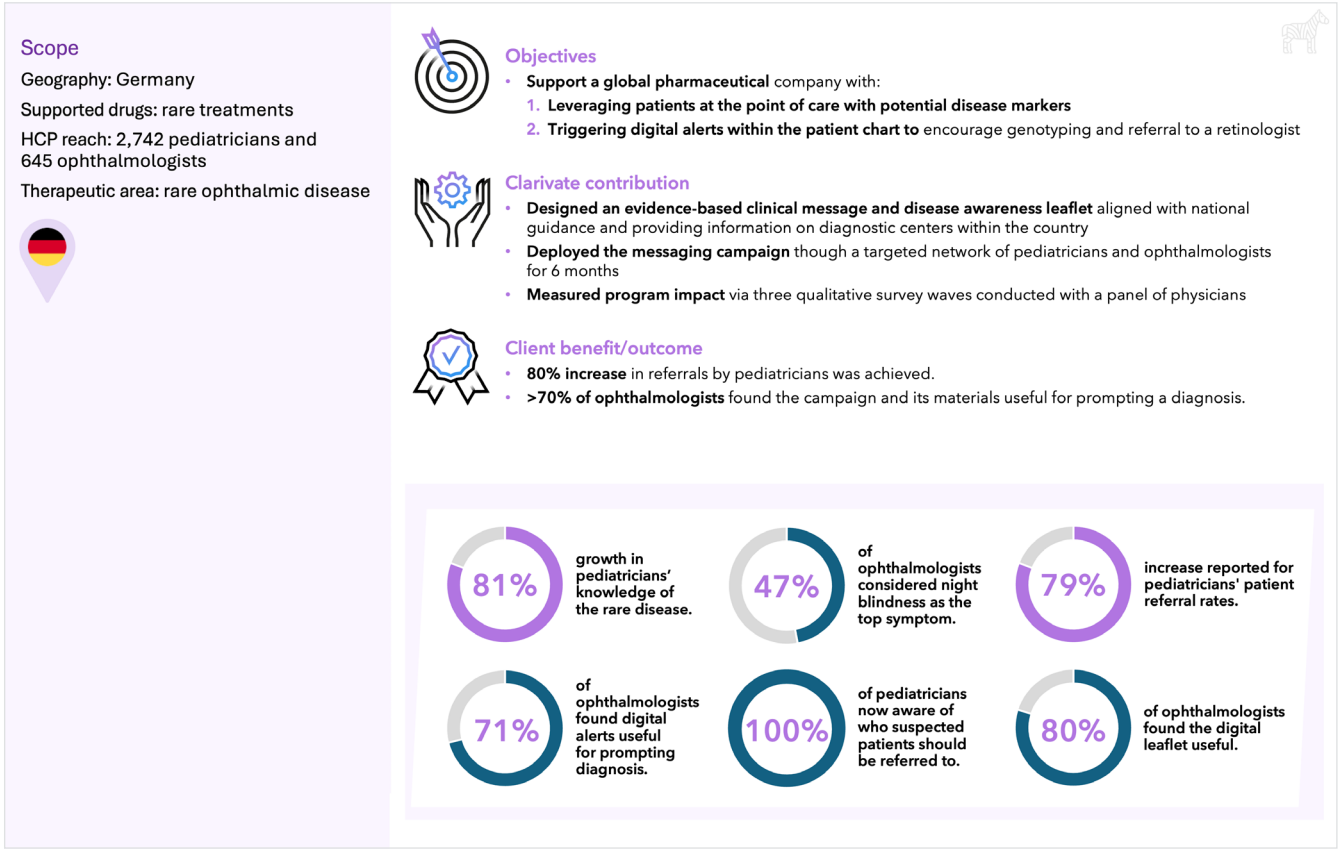
Areas of focus will vary according to the context of the condition. For those rare diseases of which there is low awareness, strategy might emphasize patient identification (e.g., AI-enhanced searches of electronic health records, family mapping tools or newborn screening), while a product launching into a more competitive space might focus on patient treatment conversion and market access at launch.

For example, one pharma launching a product for a very rare ophthalmic condition with limited treatment options in Germany focused on increasing disease awareness and patient identification using digital messaging at the point of care. This approach garnered them a better than 80% increase in referrals, with over 70% of physicians surveyed saying they found the approach useful.

70%+

of physicians surveyed said they found the approach useful.

Figure 3: Digital messaging at the point of care to drive disease awareness and patient identification



Source: Clarivate Digital Messaging at the Point-of-Care

Considering zebras from strategy to execution

Delivering value to patients with a rare disease requires a distinct set of financial and operational considerations. Successful companies in the space typically house a dedicated vertical unit with a purpose-built go-to-market strategy. Examples of this include BMS, with its cell and gene therapy unit, or AstraZeneca, with its acquisition of Alexion Pharmaceuticals, as well as companies like Vertex Pharmaceuticals that are wholly focused on rare diseases.

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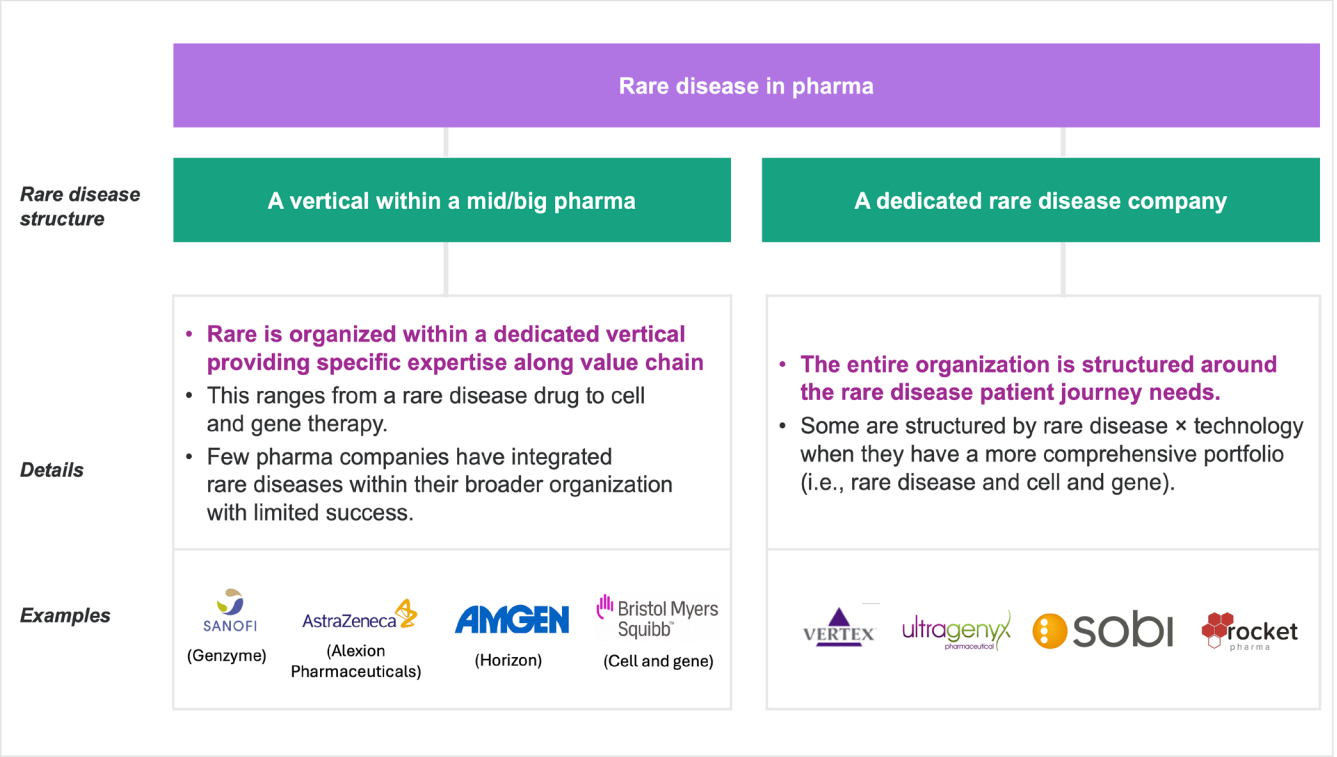
Figure 4: Pharma company rare disease models



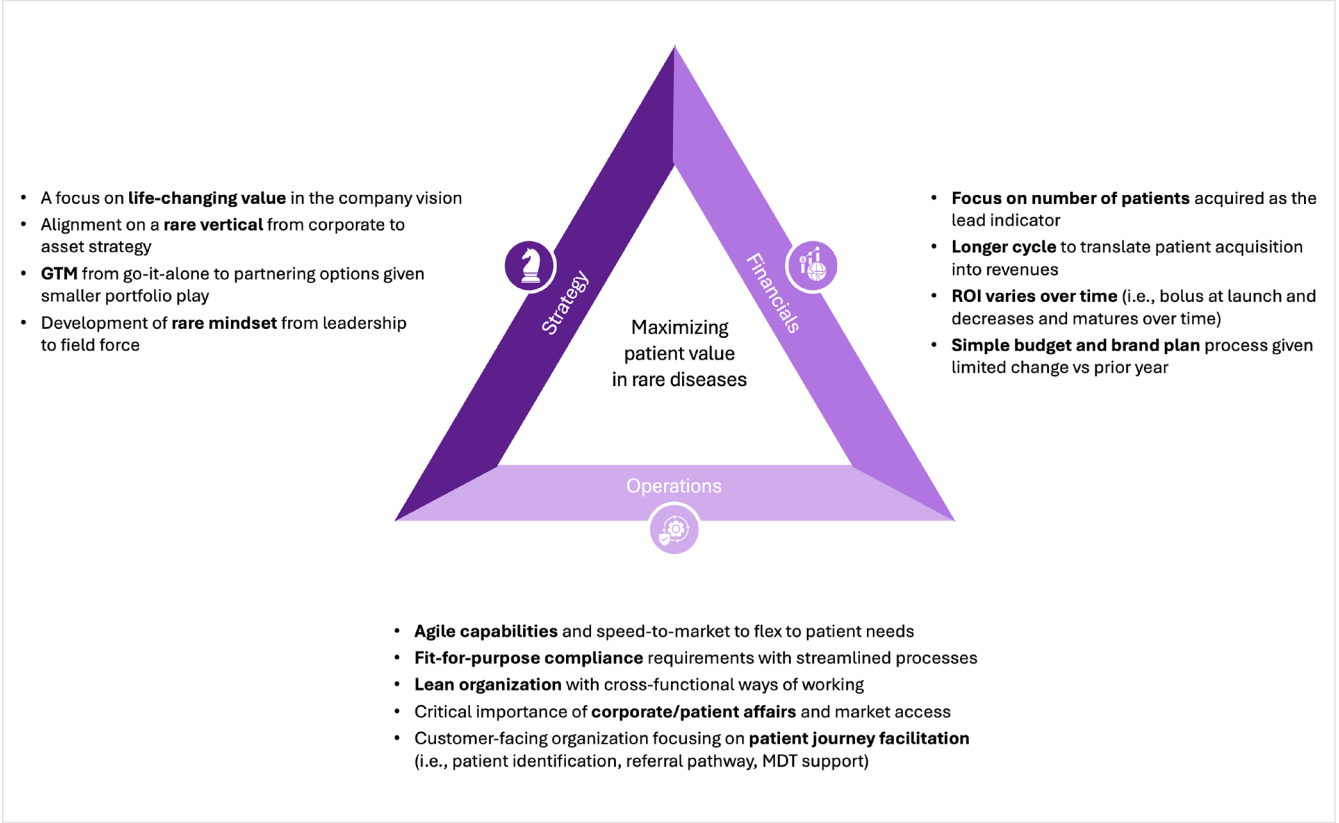
Source: Clarivate Analytics

The financial models of rare disease companies and verticals focus on number of patients as the lead indicator within the context of a longer lead cycle to translate patient acquisition into revenue. Revenue patterns may differ markedly from those of conventional drugs, often launching with a bolus of long-underserved patients followed by a drop in patient acquisition, before settling into steady growth and maturation.

Factors for success in this space include a lean, agile organization that works cross-functionally and puts patients first, as well as a fit-for-purpose compliance operation. Corporate and public affairs departments play critical roles in shaping policy and the market, while more customer-facing functions focus on project-based activities supporting the patient along their journey.

Rare disease treatments often address long-undertreated patient populations. Market-shaping efforts are critical to execution. Companies in this space have been experimenting with patient-centered approaches, incorporating patient identification, disease awareness programming, partnering with patient groups, global KOL network activation, early access program roll-out and evidence generation. In-house expertise, coupled with a high-touch approach to patient support and services, helps to facilitate innovative access models and targeted omnichannel communications, delivering value to patients and healthcare professionals along the treatment journey.

Figure 5: Rare disease innovators are structured differently from strategy to execution



Source: Clarivate Analytics

Reflecting zebras from insight to impact

The complexity of the rare disease ecosystem requires a different approach to measuring and ensuring impact. With stakeholder interaction focused on engagement and solution co-creation within the context of multi-specialty-driven pathways, innovators in this space must strike a balance between insight generation and appropriate levels of granularity.

For example, one pharma company serving patients with cystic fibrosis set out to improve medication adherence in the U.S. using an innovative omnichannel approach. They first identified patient omnichannel preferences and then mapped those insights and prioritized channels accordingly, enabling them to develop bespoke digital tools to help non-adherent patients stay on treatment.

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Figure 6: Taking a digital approach to improve adherence in cystic fibrosis

Scope **Geography:** U.S. **Audience:** Patients with cystic fibrosis **Language:** English **Therapeutic area:** cystic fibrosis

Objectives

- **Understand the individual channel adoption** among patients to:
  1. **Identify** key channel adoption trends across the patient journey
  2. **Uncover** digital channel and content preferences across the treatment journey
  3. **Determine** the tools to help the high number of non-adherent patients become adherent to treatments

Clarivate contribution

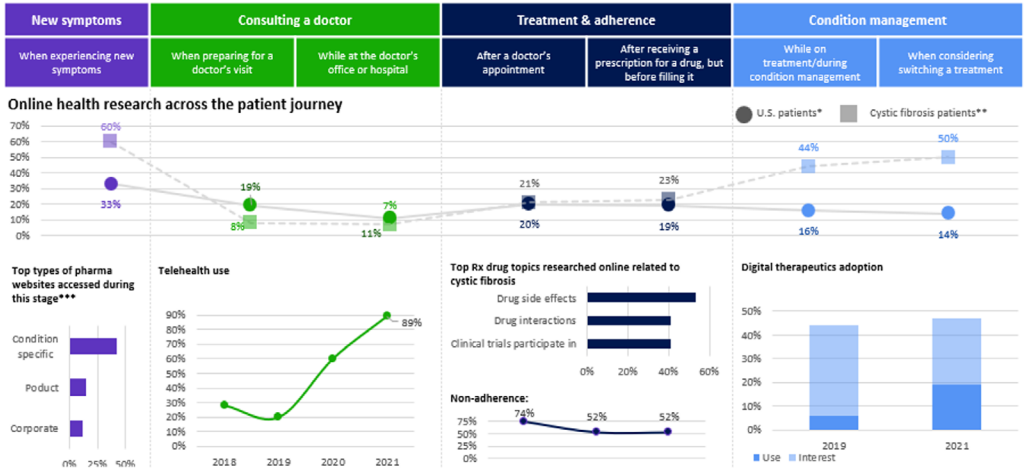
- **Mapped patient perceptions, circumstances, opinions and triggers**
- Applied Clarivate's **social intelligence and informational ecosystem mapping** methodologies combined with primary market research
- **Surfaced key insights on the key digital channels and content topics preferred by patients**

Client benefit/outcome

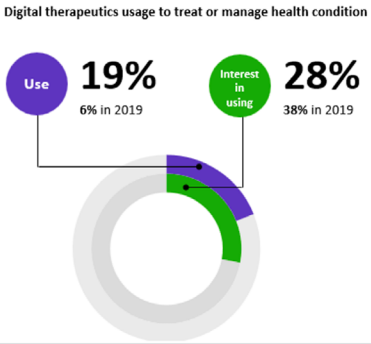
- **Uncovered that 1 in 2 patients continue to be non-adherent to treatment,** and 1 in 2 patients are non-compliant after filling their prescriptions
- **Mapped tools pharma can provide to support medication adherence,** such as calendar reminders and quick information nuggets, as the adoption of digital tools was high among the group
- **Identified opportunities for patient apps, treatment-specific content and key digital resources to support** patients' informational needs pre-, during and post-consultation

✓ Used proprietary syndicated primary market research methodology and social intelligence to map information-seeking behaviors across the patient journey and determine how to increase adherence and compliance

Patient journey summary



DTx use has increased by 13% since 2019; Cystic fibrosis patients are using digital support tools more since the pandemic



Cystic fibrosis patients agree*

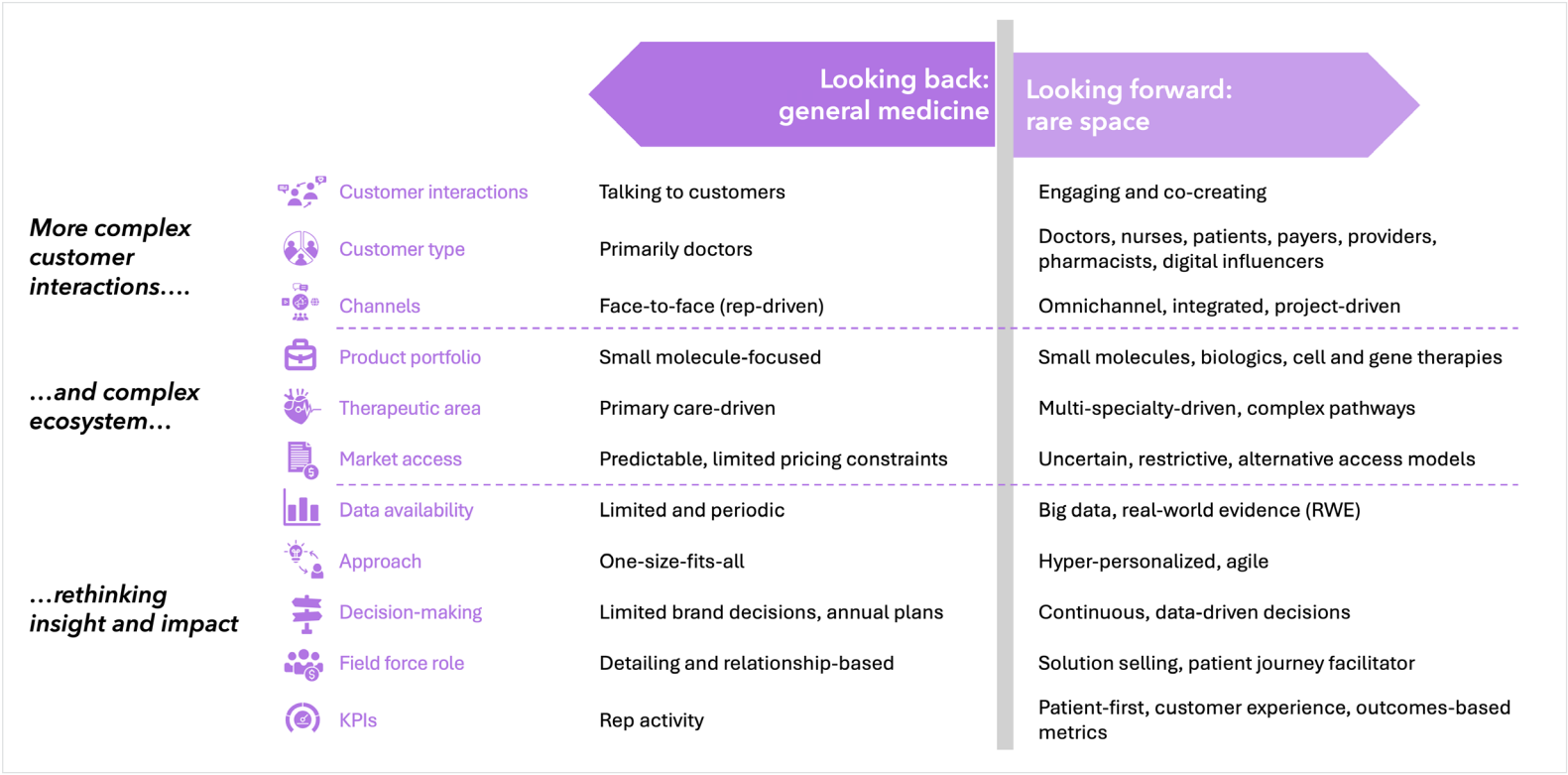
67%

Digital support tools (e.g., mobile apps, videos) for my health management became **more important now due to COVID-19**

Source: Clarivate Omnichannel Insights

Measuring impact in the rare disease space requires a patient-first approach, with KPIs encompassing brand uptake and perception (patient share, intent to prescribe) as well as commercial execution (share of voice, quantity and frequency of physician engagement) and patient identification and conversion (numbers identified, referred and treated; patient adherence).

Figure 7: The rare disease space requires a different type of engagement from insight to impact



Source: Clarivate Analytics

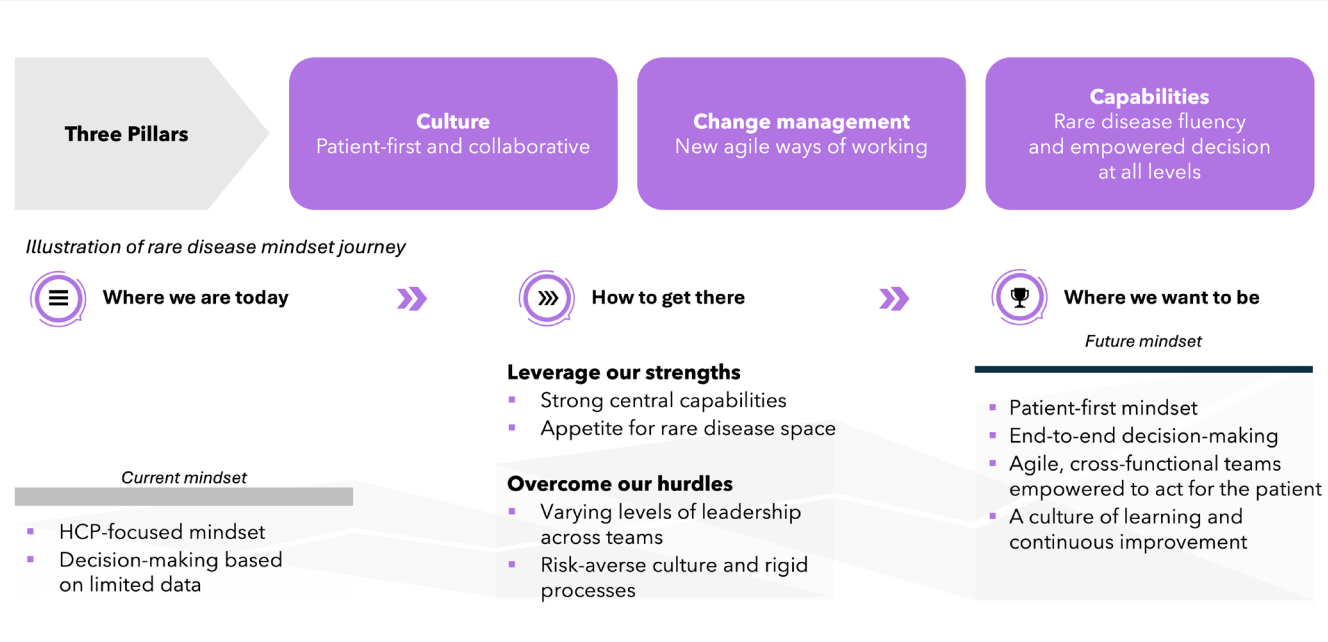
Adopting zebras as a rare disease mindset

Putting patients first calls for the adoption of a rare disease mindset — one that restlessly seeks out new ways of engaging patients and implementing solutions. This mindset rests on three pillars:

1. **A rare disease culture of collaboration and putting patients first**
2. **Change management strategies enabling agile operations and patient engagement**
3. **Rare disease capabilities with fluency and empowered decision-making at all levels**

Embracing a rare disease mindset requires that organizations critically evaluate their strategy and leverage their strengths to overcome challenges. Norgine provides a stellar example of this — CEO Janneke van der Kamp has compared mid-sized pharmas like hers to catamarans delivering "small, precious parcels" of innovative medicines to underserved patients. "Innovation in healthcare is not defined by the invention of a new molecular entity alone," said van der Kamp. "Instead, it should be defined as the development and implementation of new medical technologies, treatments, processes or care models that improve patient outcomes, enhance efficiency and reduce costs" (van der Kamp, 2025).

Figure 8: Enabling a rare mindset, by creating new models to serve the patient first



Source: Clarivate Analytics

Thinking of zebras and dazzling innovation

A group of zebras is called a 'dazzle,' alluding to the way the animal's stripes can seem to blur and confuse viewers when moving in a group. Similarly, rare diseases are often confusing to everyone involved — patients, carers, providers, researchers and innovators. Just like a group of zebras in nature, patients with rare diseases are dazzling in their own right, persevering despite the challenges they face in reaching a diagnosis and receiving appropriate treatment. In addition, the combined work of patients, healthcare professionals and researchers to reach scientific breakthroughs and enable cutting-edge therapeutics contributes to the dazzle, transforming the lives of patients with rare diseases and their carers. By fully embracing a rare disease mindset, companies can deliver on this promise and help ensure that when we hear hoofbeats, we think of zebras as well as horses.

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Laure is spearheading the Customer Strategy and Commercialisation practice within Clarivate Commercial consulting, which is spanning a range of product & customer-related areas such as portfolio & pipeline strategy and decision support, product & customer strategy, integrated patient journey, competitive landscape, commercial strategy, go-to-market model as well as launch preparation, marketing & commercial excellence or omnichannel engagement. This is supported by the robust Clarivate data, insight and expertise and global footprint with teams in the U.S., Europe and APAC.

Laure has developed her passion and expertise in rare from her professional background preparing commercial launch at global level for rare disease assets, managing country launch in the rare disease space as well as from her personal contribution to a rare disease patient association as a trustee.

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