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Medical Affairs Strategy for the Launch of Innovative Treatments Targeting Rare Diseases

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INTRODUCTION

A rare disease is typically defined as any condition that affects fewer than 200,000 people in the U.S. and < 1 in 2,000 people in the EU.[1] However, with more than 7,000 rare diseases, the total rare disease patient population reaches 400 million people worldwide, about half of which are children.[1,2] The exact number of patients with a rare disease is challenging to calculate (and is most likely underestimated) due to the difficulty of diagnosing, tracking, and defining a rare disease. Often, rare diseases are genetic and many are chronic, progressive and life-threatening. Additionally, there is often a long diagnostic odyssey and many gaps in knowledge related to the patient journey and path to diagnosis.[3] Furthermore, there are few treatments available, typically no standard of care and limited guidelines (if any) to shape the treatment and care of patients with rare diseases.[3] In fact, only 5% of the roughly 7,000 currently recognized rare diseases have an FDA-approved therapy, leaving thousands of conditions without a treatment.[4]

While rare diseases are diverse, more than 80 percent of these conditions have a known genetic cause[4] and 4,000 are monogenic or caused by a mutation in a single gene,[5] making rare diseases attractive targets for cell and gene therapies. In fact, there are 2,024 gene therapies in development around the world with approximately 50 percent of this research focusing on rare diseases.[6]

Due to the Orphan Drug Act and other incentives to focus on rare disease drug development, a shift has occurred with about 30% of the medicines in the worldwide drug development pipeline now focused on rare diseases.[7] While most orphan drug development was supported by small biotech companies in the past, by 2018, larger pharma had developed or acquired about half the new drugs approved by the US FDA for orphan indications.[8] This increased attention on drug development for rare diseases along with the diversity of organizations shepherding this development means that Medical Affairs (MA) organizations of all sizes need to adopt a different mindset and tailor their strategic acumen and launch capabilities to supporting therapies targeting rare diseases, many of which will be innovative cell and gene therapies.

This article seeks to outline the different aspects of MA strategy and launch excellence specific to supporting the development and launch of treatments targeting rare diseases and to provide a roadmap for MA teams and organizations undertaking this planning. While each rare disease is different and will require a tailored approach, this roadmap provides broad considerations for MA organizations.

THE CHALLENGES OF MEDICAL AFFAIRS IN RARE DISEASE

The challenges of MA in rare disease often include frequent strategic reprioritization of limited resources due to rapidly changing and shorter timelines related to uncertain progression of clinical drug development and regulatory authority designations (e.g., Fast Track, Regenerative Medicine Advanced Therapy). Due to a smaller overall patient/customer population, many MA teams are asked to develop and execute the launch plan with fewer people and financial resources than teams working in more common disease areas. Lack of resources and accelerated timelines mean that often MA professionals in this space wear many hats, accomplishing a range of activities that would be owned by separate functions in larger organizations while also being nimble to adapt to timeline changes. With cell and gene therapies, MA personnel often need to strategize for both the rare disease therapeutic areas and the novel therapeutic modality, which adds a layer of complexity to planning and execution and often requires internal advocacy to secure buy-in for early landscape preparation. There is the added challenge of working with little precedence due to the small number of cell and gene therapies approved by regulatory authorities.[9,10] In assessing the disease landscape and patient journey as part of strategy development, MA teams in rare disease may discover more gaps than answers, requiring creative information gathering and augmented evidence generation (e.g., registries, safety surveillance databases, claims databases and regular clinician and patient insights).

Given the limited number of patients with rare diseases, the healthcare professionals (HCPs) that treat these patients are also few. The complexity of recognizing symptoms of these diseases and the multidisciplinary care needed often results in HCPs of varied specialties being dispersed across different points of the patient journey. As different healthcare specialties may be involved in diagnosing the disease, treating the disease, and administering the therapy, the approach needs to be tailored to specific needs of different healthcare specialties and their specific roles across the patient journey. This makes thought leader mapping, scientific exchange and engagements, as well as engaging medical education programs more challenging. There may also be limited patient advocacy organizations or fragmented patient communities, which makes it very difficult for MA and their internal advocacy partners to secure the relevant patient insights to inform the strategy and launch planning.

Overall, the multiple challenges facing MA teams in supporting rare diseases often require more communication and collaborative solutions, both from the cross-functional teams internally and with the scientific/clinical/patient communities externally.

THE POTENTIAL FOR MEDICAL AFFAIRS IN RARE DISEASE

It is specifically in the challenging landscape of rare disease — difficult diagnosis, limited literature, and a dearth of treatments — where MA professionals and teams have the opportunity to make tremendous impact. Key to this impact is, 1) developing a clear strategy to ensure prioritization of the limited resources, 2) partnering with clinical development on trial design, incorporation of the patient voice, and trial recruitment, and 3) building sustainable partnerships with the rare disease community through trust, mutual respect, transparency, and regular communication. A core strength of MA is engagement and relationship building with a broad range of external stakeholders, including patients and advocacy groups, which is essential in the rare disease ecosystem. Another strength of MA is the ability to gain insights and turn insights into actionable activity that could be mutually beneficial to their organization as well as to the patient community. External stakeholder insights are essential for filling critical information gaps and are an important way to add value to cross-functional teams. Early engagement with Centers of Excellence is also valuable for identification of potential study sites and is often an important part of MA roles prelaunch. Since patients and families dealing with a rare disease often feel that their voice is unheard, MA teams can contribute their scientific communication expertise to help amplify the patient voice and fill the communication gaps by adopting a patient-centric approach in developing strategy and tactics such as clinical trial design or disease landscape assessment. Due to the high need, high impact opportunity for rare diseases currently without treatments, launching a potential treatment especially with an innovative, perhaps life-changing therapy not only provides a tremendous amount of benefit for patients and their communities, but can provide a fulfilling experience for MA professionals.

Key challenges in MA supporting rare diseases

1. Fewer people and financial resources
2. Faster clinical & regulatory timelines leading to unpredictability in launch planning
3. Need to be nimble, take on many responsibilities
4. Expertise needed on both the rare disease and often a novel treatment modality
5. Fewer scientific resources/literature and expert knowledge
6. Insights often come from more qualitative research and are not readily available
7. Fewer key therapy area/ opinion leaders
8. Education needs to be tailored to diverse specialties and audiences

BEST PRACTICES FOR LAUNCH EXCELLENCE IN RARE DISEASE

Launching a product in rare disease requires a MA organization with a pioneering, committed mindset and the individual/team disposition to work beyond narrowly defined roles. Thus, adjustments to standard launch planning and practices are required to capture the unique nuances of launching a

treatment for a rare disease and/or one that involves an innovative therapy. An example template as a starting point to understand the activities of MA in the period leading to product launch is the MAPS Best Practices for Launch Excellence Standards & Guidance.[11]

The following sections and Figure 1 provide an overview of these nuances and adjustments. Readers should recognize that each rare disease is unique, so further adaptation may be required beyond the adjustments suggested.

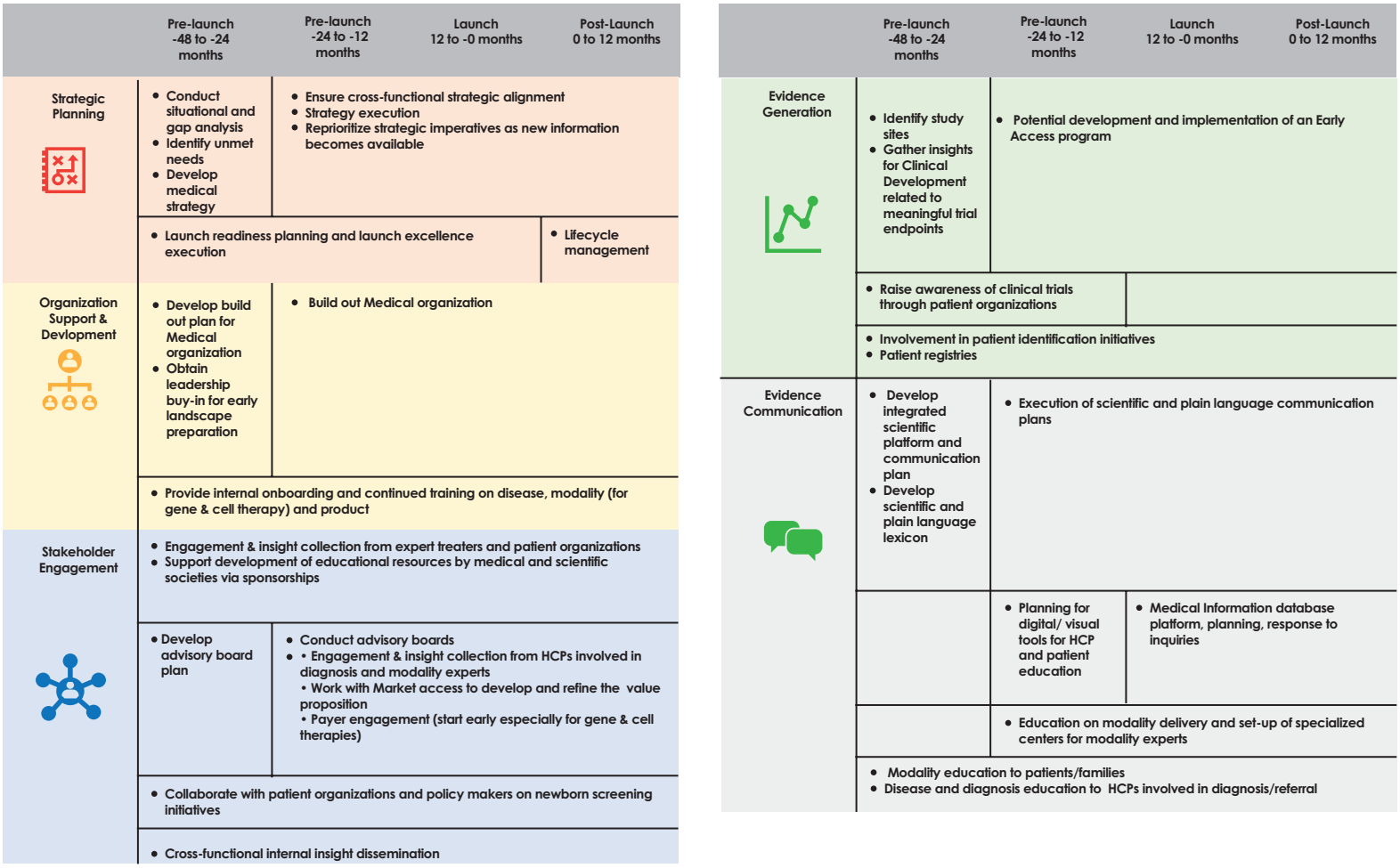

Figure 1. Medical Affairs Key Activites for Launch Excellence

Medical Strategy Development and Tactical Planning

Strategy creates purpose, efficiency, and guidance. Nowhere is this more important than when prioritizing the strategic approach and limited resources often allocated to product launch for rare disease. While conducting the situational analysis and developing the medical strategy may be daunting, especially with limited people resources, an aligned and prioritized medical strategy will be well worth the effort.

Situational Analysis

The first step in strategy development is conducting the situational analysis to ensure appropriate understanding of the therapeutic environment which will then help to identify what needs to be done to reach the desired situation. The process for conducting a situational analysis is outlined in the MAPS Medical Affairs Strategic Planning Guide[14] and focuses on four external areas including disease landscape, competitor, audience, and regulatory analyses. With more common diseases, much of this situational analysis can be completed by synthesizing information from existing sources. With rare diseases, the current environment has myriad information gaps, often including the following:

Disease Landscape: The disease landscape and natural history of disease is especially scarce. In many rare diseases there is a long diagnostic odyssey and many gaps in knowledge related to the patient journey.[3] Typically, there is no standard of care and limited guidelines (if any) with rare diseases because there are no or limited treatment options.[3] Gene therapy is often for ultra-rare (prevalence <1 per 50,000 persons[13]) monogenic conditions so even more limited disease information may be available to conduct the analysis.

Competitor: For the competitor analysis, competitors in the traditional sense are often scant due to the limited number of approved products or only products for symptomatic treatment of a rare disease, but competition in the preclinical/clinical trial space may be much more crowded. Information on competitors may be found through resources such as scientific conferences, patient organization websites, or clinicaltrials.gov.

Audience: The audience analysis is unique for rare diseases with weighted importance given to patients/caregivers/families, patient advocacy groups and policy makers. Inclusion of the patient's voice as part of the landscape assessment is critical, as these rare populations tend to be very well-informed and active in advocacy groups, and often present a compelling voice in front of regulators, payers, and clinicians. With cell and gene therapies, stakeholders with expertise in these modalities and routes of delivery must also be considered.

Regulatory: The regulatory and reimbursement analysis is challenging, especially when a rare disease involves an innovative therapy, as the regulatory landscape is still evolving, and no

playbook is set for payer engagement due to the limited number of approved therapies. The costs of these innovative therapies are high, so demonstrating the value of the treatment is of paramount for market access and is often supported clinically/scientifically by MA.

Insights gathered by MA teams from all stakeholders including clinicians, patient advocacy organizations, patients/caregivers/families, policy makers, and payers need to be collected in a systematic way to fill these information gaps and inform the development of a clear medical and cross-functional strategy.

To complete the situational analysis, tools such as a gap analysis and medical SWOT (Strength, Weakness, Opportunities, Threats) can be utilized to consolidate the learnings from the situational analysis and inform the development of the medical strategy and tactical planning. The gaps identified often lead to strategies focused initially on information and insight gathering and then a structured and integrated clinical and scientific evidence generation plan to increase the understanding of the patient journey. The medical strategy is patient-focused with rare diseases and typically involves disease awareness pre-launch and post-launch. For example, initiatives focusing on appropriate diagnosis often start early and continue after product launch. When innovative therapies are involved, the medical strategy usually also includes early education related to the treatment modality. Figure 2 provides an example of key pre-launch strategic objectives for the launch of a gene therapy in a monogenic ultra-rare disease. Since there are often limited MA resources when launching therapies for rare disease, there is a need to prioritize and maybe even weight the strategic objectives to ensure appropriate focus.

Key Takeaways/ Actions related to Medical Strategy

- Prioritize initial limited resources through development of a clear medical strategy and tactical plan
- Focus on early insight generation to supplement gaps in knowledge
- Include a broader group of external stakeholders in engagement plans
- Start early with disease awareness and earlier diagnosis initiatives
- Educate HCP and patient communities early, especially with a new treatment modality

Strategic Objetive	Pre-Launch Example
1 Diagnosis	Educate on key diagnostic criteria and testing for Disease X
2 Disease Awareness	Construct an evidence generation plan to augment and increase knowledge of Disease X
3 Treatment Modality	Provide education on gene therapy to specialized HCP treaters and the patient community
4 Efficacy/Safety	Communicate the clinical evidence of Gene Therapy X
5 Internal Launch Preparedness	Develop an internal training program on Disease X and Gene Therapy X to educate internal stakeholders

Figure 2. Example of Pre-launch Strategic Objectives for a Gene Therapy for Potential Treatment of a Rare, Monogenic Disease

Organizational Support and Capability/Competency Development

Launching a product in rare disease requires building a MA team to support the launch and ensuring that the team has capabilities/competencies to successfully launch the product. Figure 3 illustrates the experience and skills of MA professionals who are often a better fit for working in rare disease.

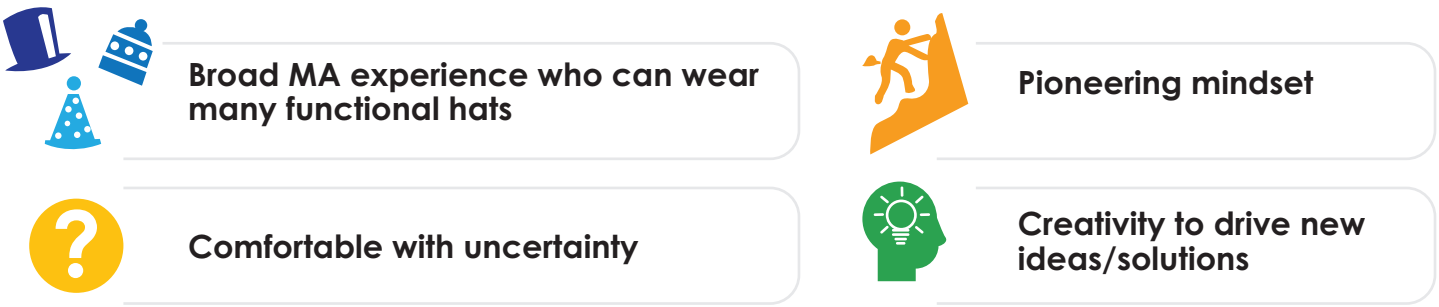

Figure 3. Desired Skills for MA Professionals Working in Rare Disease

There is an expectation of high and sustained engagement with major stakeholders and nimbleness in the rare disease space which is often better suited for a small sized company. As larger pharmaceutical companies with more entrenched and less malleable structures continue to move into the rare disease space, they will need to alter their approach to achieve this nimbleness (e.g., by establishing a rare disease division).

When building a MA organization for a company's first launch, MA leadership will initially be responsible for strategy development, execution of plans, and organizational buildout. Providing education related to the roles of the various MA functions along with a proposed scale-up of MA aligned with clinical development timelines to internal colleagues may be helpful for obtaining buy-in from company leadership to start landscape development early.

Building a MA organization for rare diseases must be done thoughtfully and the approach may vary depending on company size, disease, and if an innovative therapy is involved. One approach to capability building in rare disease is to align capability expansion with data availability in which each phase of development coincides with incremental capability growth (Figure 4).

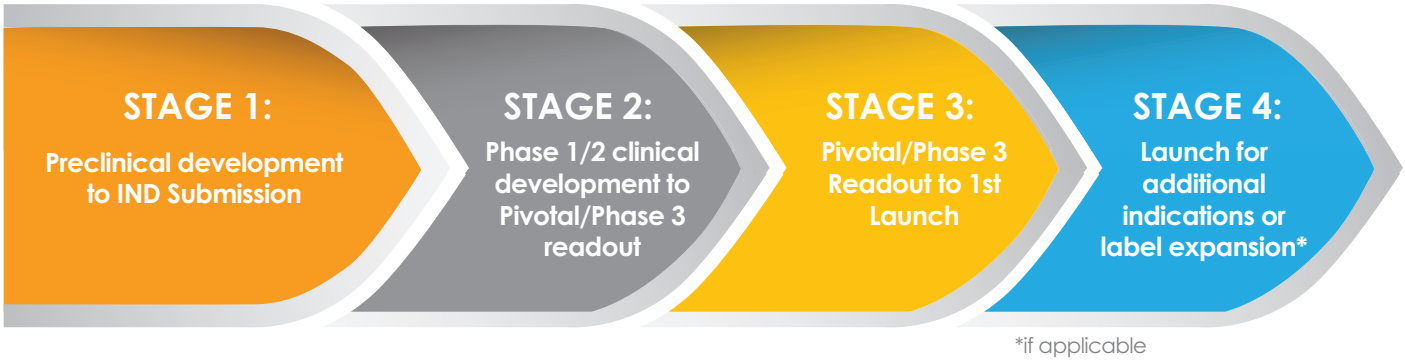

Figure 4. Potential Stages of Building a MA organization in Rare Disease

When treatments involve innovative cell and gene therapies, alternative approaches to resource planning such as use of agencies and/or contractors may be needed in the early stages to ensure on-time execution of MA plans due to the following factors. With cell and gene therapies, important data readouts occur even with a very small number of patients, well before the pivotal data readout. MA personnel must also be staffed to start educational activities related to disease awareness as well as the novel therapeutic modality at an early stage. A robust onboarding program and internal continued training/education are needed, especially with cell and gene therapy, because these modalities are rapidly evolving from both a scientific and technological standpoint.

Internal cross-functional collaboration and communication between MA, clinical, patient advocacy, commercial development/marketing, and market access are critical to ensure we are providing consistent communication to all key external stakeholders as all of the stakeholder groups in the rare disease community are highly connected and also have limited bandwidth. Collaborative solutions, rather than siloed approaches, are required to best serve the rare disease community and MA can often help to drive these collaborations as they are often the center of the interactions.

External Stakeholder Engagement and Insight Gathering

n the rare disease space, early engagement with a broad set of traditional and non-traditional external stakeholders is needed with a greater emphasis on patients/ patient organizations as stakeholders (Figure 5).[3]

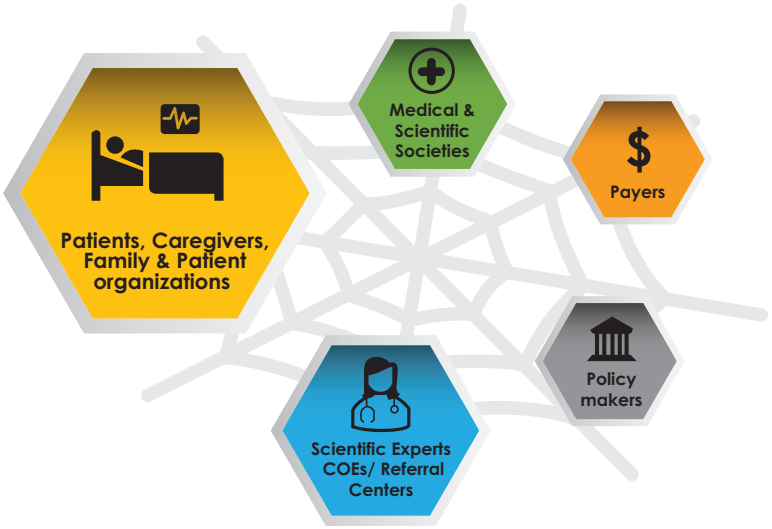

Figure 5. Key stakeholder Importance in Rare Disease

The needs of any one stakeholder group are highly connected to those of other stakeholders in the rare disease community, so MA must become an integrated member of the community and be seen as authentic, transparent, trustworthy and committed to driving positive change and adding value for all key stakeholders. When launching in the rare disease space, MA must take the needs and perspectives of all these stakeholders into consideration. Here, we provide a brief overview of the key stakeholder groups in most rare disease communities.

Patients, Caregivers, Family & Patient Organizations

Imagine launching a treatment in a new disease space. What issues would this treatment need to address? MA professionals in rare disease have the opportunity to ask this question and, increasingly, the answers come from patients or their family members/caregivers as many rare diseases occur in children. This requires very early engagement with patients/caregivers/patient organizations to understand the patient journey and diagnostic odyssey, identify educational/information needs, get feedback on meaningful trial endpoints, outcome measures that matter the most, burden and challenges of living with the condition, concerns about clinical trial participation and eventually raise awareness of the disease and clinical trials.[3,14] In some ultra-rare diseases, there may not be established patient organizations, requiring innovative approaches to connect with patients. Many rare disease patient and caregiver communities stay connected through social media, so conducting social listening of patient conversations is an easy way to indirectly learn about patients and caregiver concerns, unmet needs, how patients manage their disease, and interact with HCPs[8].

MA collaborates more with internal Patient Advocacy departments in rare disease relative to other specialty/general medicine areas and Patient Advocacy may even be a function within MA at some companies. MA partnership with Patient Advocacy is important to complement their outreach with insight gathering and patient appropriate scientific communication.

Scientific Experts/Centers of Excellence/Referral Centers

In rare disease, a wide range of HCP engagement is necessary in order to gather insights related to the patient journey, diagnosis, and therapeutic modality. There are typically only a small number of Centers of Excellence and HCPs who are highly specialized experts on the disease and are currently treating patients or are part of a clinical trial[3, 27]. However, some rare diseases may be without an existing scientific community, requiring MA teams to engage with the "closest experts" to grow collaborations and eventually identify patients. Early engagement with the highly specialized experts especially those at academic centers who have done the initial research on the rare disease of interest, can become extremely helpful in the fight against a disease by acting as thought leaders, trial investigators, advisory board members, and much more. Insights from these experts (early and often) may guide key study design considerations such as whether the endpoint is measurable and whether the endpoint is acceptable proof of therapeutic efficacy. These experts may also provide valuable insights related to the patient journey.

In addition, there are HCPs who are less knowledgeable about the disease but are important because they may be involved in the diagnostic journey and referral of patients to the disease experts/Centers of Excellence.[1,15] These HCPs are important to focus on for disease awareness education. They may also provide valuable insights related to how to educate more broadly on the disease and can be engaged in peer-to-peer education, so non-expert HCPs understand the impact, and set-up referral structures.

With cell and gene therapies, MA teams may need to broaden their engagement and relationship building efforts even further to include experts in treatment modalities, specialized surgical techniques, or route of administration/delivery. For example, for some neurodegenerative diseases, gene therapy is administered using invasive brain surgery and centers need to be specialized and set-up appropriately to ensure access.

Policy makers

MA professionals working in the rare disease space may be involved with policy efforts that advance the development of treatments, diagnostic opportunities, and access. For example, to get a disease included in the federal U.S. newborn screening panel (i.e., recommended uniform screening panel or RUSP), extensive requirements must be met such as the availability of appropriate tests and treatment and demonstration of benefit from early intervention.[16] After a disease is included on the RUSP, immense effort is needed to get the disease on state level newborn screening panels. To make things even more complex, the process and requirements for getting a disease on newborn screening panel varies from country to country. MA professionals along with patient organizations, scientific experts, and policy makers may be involved with early newborn screening initiatives such as assay development, pilot programs, genetic testing, and education/awareness.

With cell and gene therapies, MA teams may need to broaden their engagement and relationship building efforts even further to include experts in treatment modalities, specialized surgical techniques, or route of administration/delivery. For example, for some neurodegenerative diseases, gene therapy is administered using invasive brain surgery and centers need to be specialized and set-up appropriately to ensure access.

Payers

Historically, payers were often less likely to push back on price for rare disease therapies due to their lower total budget impact, but this is changing especially with the emergence of high-cost cell & gene therapies. As a result of increasing payer scrutiny, there is a trend towards tighter cost controls and the need for creative reimbursement strategies.[8,9,17,18] MA will need to support colleagues in Market Access and HEOR to make a compelling case for the value of a therapy through integrated evidence planning, generation and dissemination. One challenge is the fact that payers often do not have existing knowledge about some rare disease (the rarer the disease, the less they are likely to know), so education will be required about the disease's natural history, the level of unmet need, study endpoints and their relevance to patient benefit. Early input from payers may help organizations ensure that data coming out of trials is sufficient to enable coverage decisions and may help understand payers' expectations around durability of benefit and long-term outcomes. Responsibilities of MA related to payers may vary by organization. MA may be responsible for payer engagement and insight gathering in line with country guidance19 or may have a more supportive role in developing the value proposition for the therapy.

Key Takeaways/ Actions related to External Stakeholder Engagement

- Engage early and target a diverse set of stakeholders
- Gather insights to help fill in the gaps in the patient journey
- Engage with both HCPs who treat the disease and those who diagnose patients
- For cell and gene therapies, engage with modality/administration experts, if applicable
- Start early with newborn screening efforts and work in collaboration with patient organizations
- Support development of a robust value story for cell & gene therapies to support reimbursement
- Support development of educational resources by medical and scientific societies via sponsorships

Medical and Scientific Societies

MA may be able to partner with scientific societies on some of the needed activities of education and communication, and to build awareness and presence in the HCP landscape. Often, CME accredited or non-CME medical education, and sponsorships can be provided through these societies as they are trusted sources of clinical and scientific information. For example, the American Society of Gene & Cell Therapy (ASGCT) has developed free training modules for patients on gene therapy modalities.[20] Also, scientific societies within disease spaces may have existing outreach/education resources, such as the HCP-facing materials produced by the International Society of Thrombosis & Haemeostasis (ISTH).

Evidence Generation

As we've seen, in most rare diseases, a primary challenge is lack of knowledge about the disease itself and no/limited standard of care. With this in mind, evidence generation activities seek to create disease knowledge and a company's product may eventually become the standard of care. The focus of evidence generation 2-4 years prior to launch is typically on progressing the overall clinical development program and starts with early input from thought leaders, payers and patients/patient organizations, with the goal of identifying study endpoints that are meaningful to patients and caregivers and ensure trials are not too burdensome for patients and their families. In addition to gathering these insights, MA can also play a key role in preliminary feasibility assessments of Centers of Excellence to support the Clinical Team in identifying study sites. MA may also need to assist with or drive 'patient finding' activities related to clinical trial recruitment. Early engagement with patient organizations to help 'find' potentially eligible patients is critical as they can disseminate clinical trial information to people living with the disease to help them understand the therapy and if they are potentially eligible for the trial. MA may also be involved in conducting broad educational efforts to identify rare patients earlier in their treatment journey or using sophisticated algorithms to identify patients from claims databases.[8,18]

Due to small number of patients with rare diseases, evidence generation may need to make use of experience-based and qualitative studies or real-world evidence to build the patient journey and increase understanding of the burden of illness and healthcare resource utilization (Figure 6). With some rare diseases, especially when an innovative therapy is used, randomized controlled trials are not possible and robust natural history study data is needed as a comparator to demonstrate a therapy's potential impact. Due to limited patient numbers, real world data is often used to characterize the typical natural history of the disease.[8,21]

Figure 6. Primary sources for evidence generation in rare disease Beyond registrational trials

Patient registries are important in the rare disease space for a variety of purposes. Registries may be a helpful resource for understanding the natural history of a rare disease. These registries are also often required for post-launch evidence generation to fulfill regulatory requirements and are especially important with gene therapies where extremely long follow-up times (as long as 15 years) are required.[22] Typical phase 4 and investigator-initiated studies done as a part of lifecycle planning are extremely challenging in the rare disease space due to the small number of patients, so patient registries are often the only option for continued evidence generation. Collaboration with scientific or advocacy organizations are often the best route to get these registries in place, although this approach may not be an option for registries required as part of a regulatory commitment to build real world safety and efficacy. Significant financial resources and in-house expertise with registries are also required.

Development and implementation of Expanded Access programs for individual patients ahead of a regulatory approval may become an important consideration for MA professionals in rare disease, especially when clinical trial participation is not possible and there is no alternative therapeutic option. Although the primary intent of Expanded Access remains providing treatment to patients, these programs may be a source for additional data generation depending on various factors such as country regulations and design of the program.[23]

Evidence Communication

Development of an integrated medical communications strategy and plan is especially important in rare diseases where communication with diverse audiences is necessary, and the recent MAPS white paper provides a helpful guide.[24] MA is used to speaking the language of scientific exchange. However, the intimate involvement of patients, caregivers, and advocacy groups and other non-scientific members of a rare disease community means the function also needs to develop materials and communications written in plain language.[25] To develop effective and compliant patient/caregiver-focused communications, MA needs to first understand the patient needs and concerns as well as the country regulations governing these communications. Also, prior to development of patient/caregiver-focused communications, development of a plain language lexicon that is married to the HCP-focused scientific communications platform and lexicon is a helpful starting tool.

Due to the lack of understanding of complex therapies (e.g., cell and gene therapies) or those with a new mechanism of action (e.g., disease modifying versus treatment of symptoms), more education about how the therapies work and the risk:benefit profile may be needed. Educating patients/caregivers can empower collaboration between patient and provider in a model of shared decision-making concerning an eventual treatment, which should be considered when planning publications.[14,26] When developing publication plans, MA should include plain language summaries to provide patients/caregivers access to key data on new products. In addition, many rare patient and caregiver communities stay connected

through social media, so communication strategies using social channels should be considered for education and awareness activities as long as appropriate precautions are in place to ensure compliance.

With rare diseases involving innovative therapies, there is a need for more tailored HCP education with smaller target audiences. For example, gene therapy thought leaders may need education on the modality, how it is delivered, and how to set up a specialized center, whereas broader specialists may need education on how to diagnose the disease or when to refer. In addition, because these clinical trials involve a small number of patients and sites, those investigators that gain clinical experience with a therapy during the trials will be important educators to share their experience with a broader audience of treating specialists.

In addition to more traditional education tools (e.g., continuing medical education, symposia at medical conferences, expert speaker programs, and journal articles), visual and digital resources may be helpful in describing complex mechanisms of disease or mechanism of action of the therapy. MA teams launching innovate therapies may need to reprioritize the creation of these innovative communication tools from "nice to have" to "must have" early in the planning process. For example, Spark Therapeutics used plastic vector models as gene therapy teaching tools and the free app Turning Genes into Medicine to help visually explain gene therapies in an easy-to-understand way. These types of initiatives can be very helpful to HCPs that have a low level of comfort in discussing gene therapy with their patients.[27]

Due to increased involvement from the disease community and lack of approved treatments for rare diseases, there is a greater demand for rapid data communication which results in less time for analysis and external expert feedback prior to data dissemination. MA professionals need to be agile with data interpretation. Use of virtual advisory boards or having a standing steering committee of external experts can help to facilitate rapid external feedback.

Key Takeaways/ Actions related to Evidence Generation, Dissemination, and Communication

- Explore innovative patient finding initiatives to help with trial recruitment
- Build expertise with real world evidence and patient registries
- Include plain language summaries of key data as part of the publication plan
- Explore digital resources for explaining complex mechanisms
- Be prepared for rapid data communication

CONCLUSION

MA strategy for the development and launch of treatments targeting rare disease is built on the foundation of best practices for medical strategy development, tactical planning, and launch excellence, but with significant adjustments tailored to the rare disease and treatment modality the MA team is supporting. Designing a scalable and customized MA organization optimally resourced to execute on strategic launch plans is a critical component. MA in rare disease is on the cutting edge, requiring individuals and teams to be nimble and to be able to forge a path into largely unknown territories. However, overcoming challenges in these areas also has the potential to deliver great benefit, both to patients in the rare disease community and also to MA professionals who step into this space with intelligence, intention and a passion for making a real difference in real lives. In this way, MA in rare disease feels even more like a true collaboration between our area of the pharmaceutical industry and the communities of patients, providers, researchers, payers, and policy makers that seek to provide sustainable benefit in a climate of great need.

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