

Virtual 16th Annual Meeting of ISMPP

The Evolving Role of the
Scientific Communications
Professional in an Open World



June 16-18, 2020 | Presented Virtually



Optimizing Medical Communications for Rare Diseases

Release Date: June 17, 2020 | Expiration Date: June 18, 2020 (Live); August 31, 2020 (Enduring)

Estimated time to complete activity: 40 minutes

Jointly provided by Postgraduate Institute for Medicine (www.pimed.com) and ISMPP (www.ismpp.org)



Target audience and Learning objectives

Target Audience:

- This activity is intended for medical publication and medical communication professionals engaged in transmitting evidence-based scientific information to healthcare professionals, payors, patients and the public

After completing this activity, the participant should be better able to:

- Tailor scientific platforms and plans for publications and medical affairs for rare diseases
- Identify innovative ways to reach audiences
- Leverage patient-level data to tell the scientific story
- Use disease state education to expand the rare disease community

Educational credit



JOINTLY ACCREDITED PROVIDER™
INTERPROFESSIONAL CONTINUING EDUCATION



Continuing Pharmacy Education

- In support of improving patient care, this activity has been planned and implemented by the Postgraduate Institute for Medicine and ISMPP. Postgraduate Institute for Medicine is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.
 - **For additional information and instructions on how to obtain credit please refer to:** <https://ismpp.memberclicks.net/continuing-pharmacy-education-participation>

ISMPP CMPP™ recertification credit

- This activity has been approved for .5 ISMPP CMPP™ recertification credits

Faculty



Scott Baver, PhD
Senior Director,
Medical Affairs



Jeffrey Stumpf, PhD, ISMPP CMPP™
Scientific Director
MedThink SciCom



Disclaimer and disclosures

- The opinions expressed here are those of the individual panelists and do not necessarily reflect those of employer(s) past or present or ISMPP
-

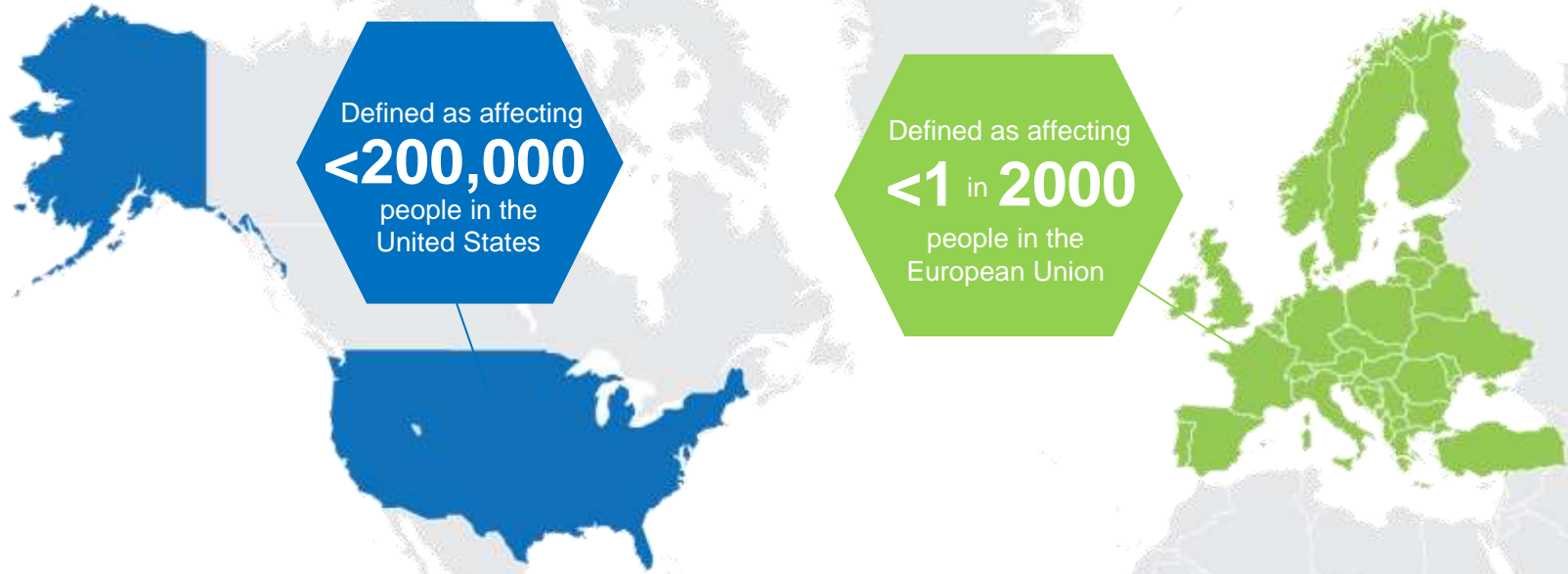
In relation to this educational activity:

- The faculty have nothing to disclose
- The PIM planners and managers have nothing to disclose
- The ISMPP planners and managers have nothing to disclose

In Rare Diseases...

**If you've seen one patient,
you're an expert. If you've
seen two patients, you're
a world-renowned expert.**

Rare Diseases: By the Numbers



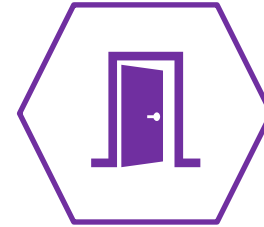
Estimated 7000 rare diseases affecting 25-30 million Americans

Common Aspects of Communicating About Rare Diseases



CHALLENGES

- Low awareness among general practitioners (or even specialists)
- Difficult to correctly diagnose
- Inconsistencies in who treats patients and how patients are treated



OPPORTUNITIES

- High engagement with KOLs
- Patients are knowledgeable and involved in their healthcare decisions
- Active advocacy organizations

It is important to tailor medical education to rare disease audience, but reaching that audience may require innovative and nuanced approaches

Learning Objectives

1

Tailor medical affairs communications strategy in rare disease

2

Identify innovative ways to reach audiences

3

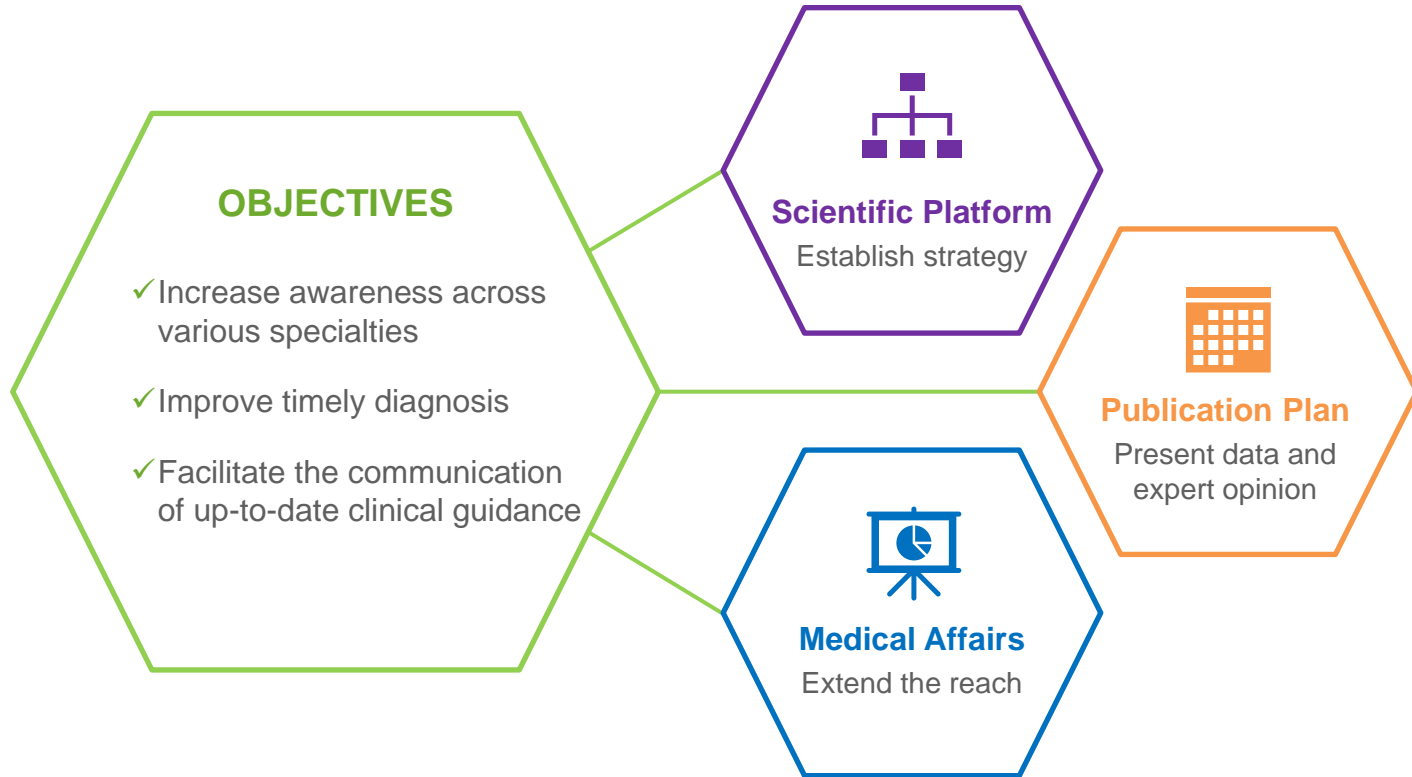
Leverage patient-level data to tell the scientific story

4

Use disease state education to expand the rare disease community

Tailoring Medical Affairs Communications Strategy in Rare Diseases

Building a Medical Communication Strategy for Rare Diseases



Aligning Scientific Platform With Rare Disease Strategy



Scientific Platform to Address Common Questions in Rare Diseases



Unmet need

- Epidemiology
- Patient population
- Burden of disease
- Burden of treatment

Who are these patients?
What are their lives like?
Which HCPs are treating them?



Mechanism of disease

- Anatomy
- Physiology
- Pathogenesis

What is the underlying cause?
Do the HCPs understand the MOD?



Diagnosis and treatment

- Diagnostic criteria and testing
- Clinical guidelines
- Treatment landscape

Who finds the patients and how?
Are treatment recommendations consistent?

Publication Plans Focus on Patient Issues and Disease State Education

Key difference in rare diseases is that its pivotal trial will be smaller than that conducted in other diseases



CASE STUDIES

- Exemplify the patient journey
- Provide opportunities for the investigator to comment on disease management
- Involve the patient more intimately in the publication process
- Establish a greater connection to the HCPs who are most engaged in the rare disease



REVIEWS

- Establish or support the current treatment and management guidelines
- Generate literature-based data through systematic reviews to establish disease characteristics, burden of disease, etc

Medical Communication Strategy Focuses on Extending the Reach

- What do various audiences need to know about the rare disease?
- How many types of specialists will initially treat a patient?
- Will they all be aware of the rare disease?
- What might pharmacists, nurses, and caregivers need to know?

How can the data tell the story of the patient journey?





Case Study: “Find the Needle in the Haystack”

Situation: Although obesity is an extremely common condition, particularly in the United States, rare mutations in genes involved in satiety pathways can cause extreme, excessive hunger and obesity

Challenge: Diagnosis is difficult because there are so few people with the rare genetic disease among so many people with obesity. Also, the clinical characteristics were assumed from the early case studies

Approach:

- Focus on known epidemiology data in the scientific platform
- Provide robust literature analyses of clinical characteristics for publication
- Provide MSL tools on recognizing the differentiating characteristics



Case Study: “Do I Need to Know That for the Test”

Situation: Choroideremia is a rare inherited retinal disease that causes progressive loss of visual acuity

Challenge: Choroideremia is not on the board exam for ophthalmologists. Also, ophthalmologists were generally not comfortable with genetics

Approach:

- Scientific platform narrowed down the key strategic topics that were needed for education of the most important audience
- Publication plan included expert commentaries on genetics of retinal diseases and differential diagnosis
- Disease state education targets specialists on the basics of physiological and genetic diagnosis through publications

Case Study: “Get With the Times”



Situation: Although pharmaceutical therapies were shown to be safer and more effective, many endocrinologists continued to treat certain pituitary diseases with radiation therapy

Challenge: Although treatment guidelines supported the use of pharmaceuticals after surgery, the field was slow to transition to newer treatment paradigms

Approach:

- Focus part of the scientific platform on strategies for discussing treatment guidelines
- Involve experts to write opinion articles in support of treatment guidelines, using available data in literature
- Provide materials (eg, leave-behinds, quick reference guides) highlighting consistencies among the treatment guidelines

Key Summary Points for Medical Communication Strategies



Understand the disease state needs and solidify the communication strategy with the scientific platform



Support the enthusiasm of KOLs to publish and present key disease state education points



Focus and prioritize disease state education strategy

Reaching the Correct Audience for Rare Diseases

Reaching the Correct Audience



**Who needs to
know the information?**



**What do they
need to know?**



**How should information
be delivered?**

Process for Incorporating Publication Amplification Into Publication Planning



- Determine high-priority publications for which audience awareness is critical



- Determine audience sources of information and preferences for learning
- Partner with third-party dissemination partners to determine options



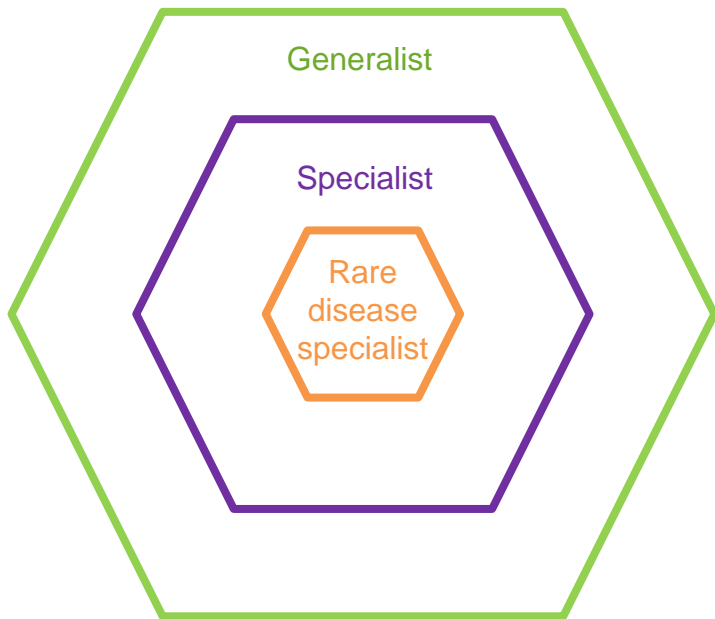
- Build any new materials needed
- Implement chosen amplification tactics



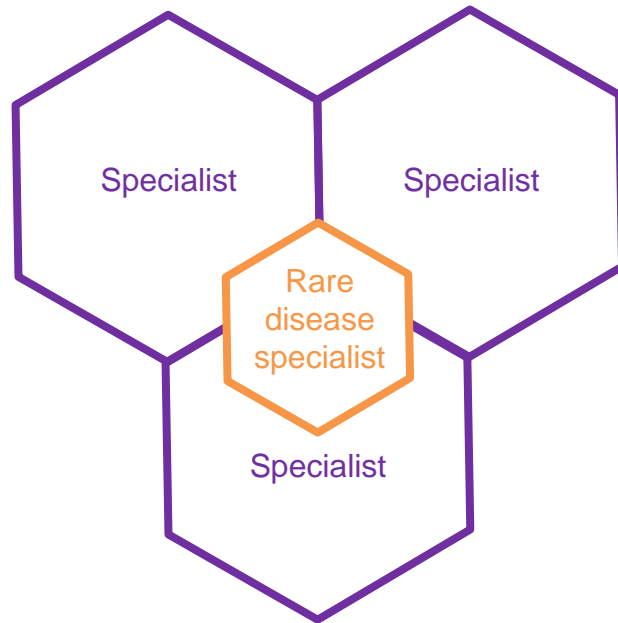
- Define and measure the success of initiatives (eg, increased article views)

Two Examples of Targeting Various Audiences According to Extent of Disease Symptoms

Localized Symptoms

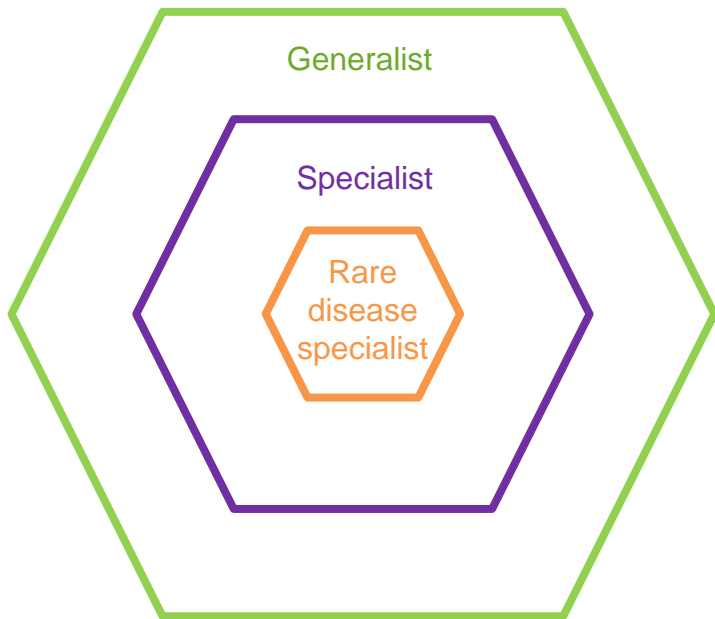


Multisystemic Symptoms



What Might the Audience Need to Know: Localized Symptoms

Localized Symptoms



Generalists: What to watch for

Example: Dentists are the first to diagnose people with acromegaly

Specialists: High-level information on disease management

Example: A general endocrinologist may be treating the acromegaly because the patient is not close to a site with an expert

Rare disease specialists: Product-specific information

Many rare disease specialists have participated in all the major trials and heavily influence treatment guidelines



Case Study: “Providing a Lifeline”

Situation: Life-threatening swelling of the larynx will cause patients with hereditary angioedema to go to the emergency room where on-demand treatment is needed

Challenge: EMTs may not recognize the symptoms of hereditary angioedema or are unable to administer the medicine, resulting in death or unnecessary surgical procedures

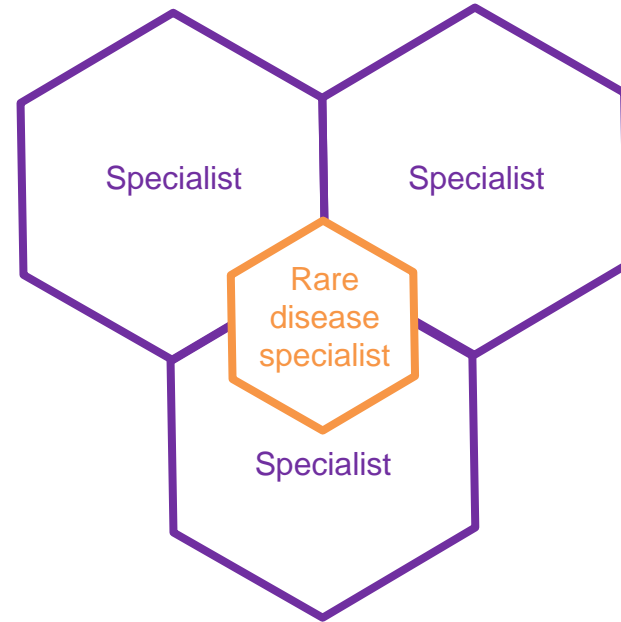
Approach:

- Quick reference guides for emergency medical care and use of on-demand medication (similar to what is done with anaphylaxis)
- A review of on-demand medications with supplemental videos

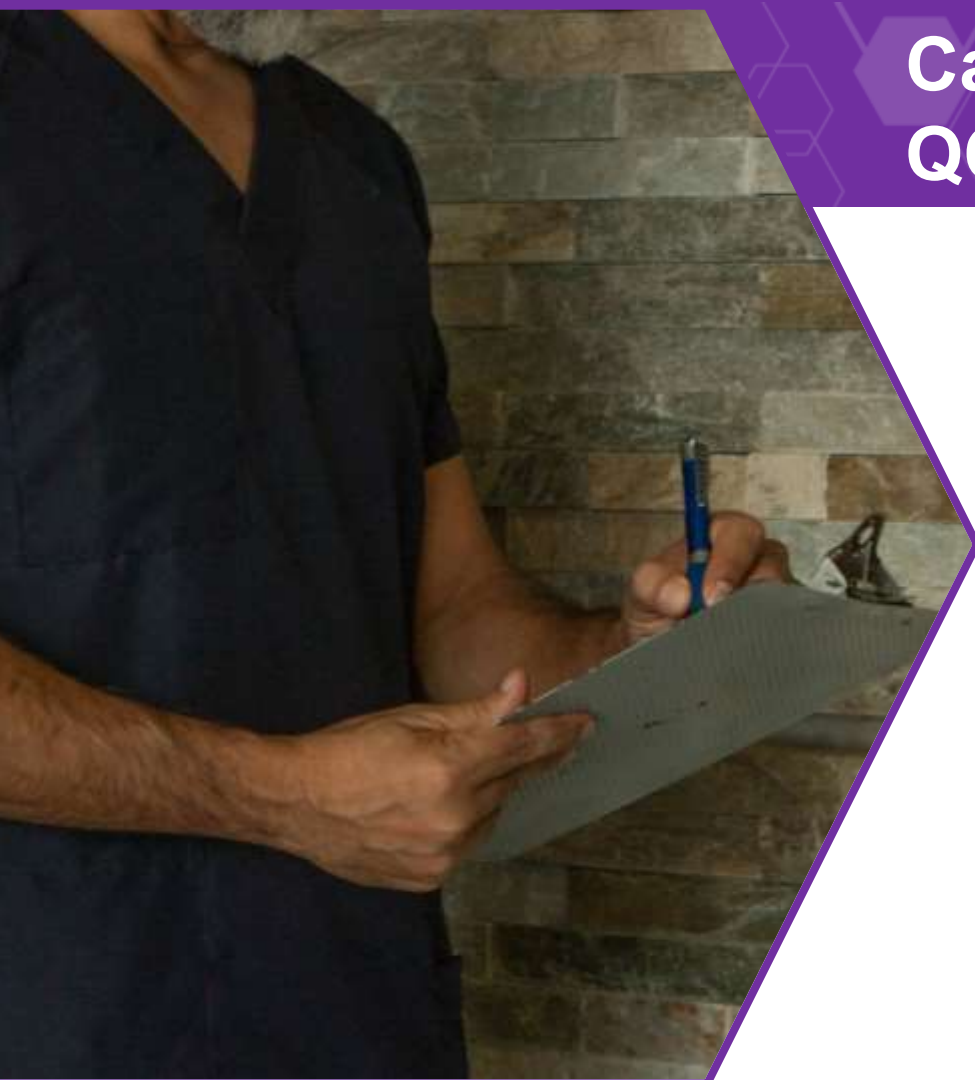
What Might the Audience Need to Know: Multisystemic Symptoms

- Some rare diseases are characterized by a wide variety of symptoms, each of which is regularly managed by a different specialist
- Therefore, multiple specialists can diagnose and be the primary treater of the rare disease
- Each specialty will need its own communication strategy so that particular audience may be reached

Multisystemic Symptoms



Case Study: “Practical QOL Assessments”



Situation: Often, QOL assessments are not tailored to rare diseases

Challenge: Physicians use inconsistent methods of assessing disease burden and progression in the patient, which can lead to suboptimal management

Approach:

- Working with experts to develop a short communication or supplemental materials featuring a physician checklist of questions to ask that are specific to the disease state and guidance for answers to focus on

How Should Information Be Delivered



Bite-sized (modular) content and repetition can increase retention



Create content for online portals where HCPs go to learn



Send post-conference emails tailored to questions asked at the Med Info booth

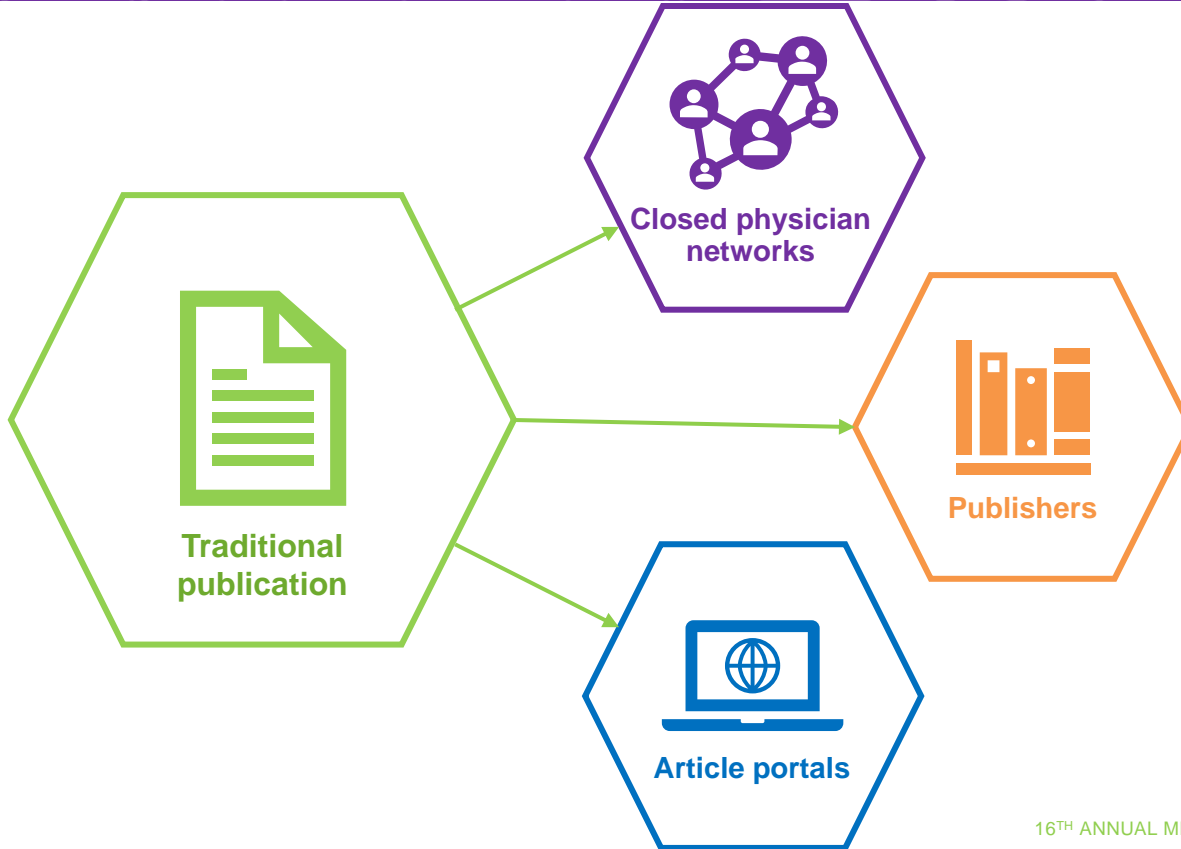


Hold educational events at professional society local chapter meetings or regional conferences



Create learning “events” (eg, in conjunction with Rare Disease Day®)

Third-Party Dissemination Partners Can Drive Readership for Key Publications



Key Summary Points to Reaching the Right Audience



Have a plan tailored to each audience



Keep the plan simple! What is the most important information to deliver to the audience?



Be innovative within the publication space and get creative in medical affairs deliveries

Leveraging Patient-Level Data

Rare Diseases Are Rare, But Not to the Patients

- For some conditions, rare diseases affect the patients every day
- Patient diagnostic and treatment journey is often only understood by the rare disease specialists (other than the patients themselves)
- Even trial data are difficult to interpret because of the small size and unconventional or unfamiliar endpoints

Communicating data from an individual perspective will increase awareness and address knowledge gaps





Case Study: “The In-Between”

Situation: In genetic diseases that cause periodic painful and debilitating swelling events, the burden for the patient goes beyond hospitalization utilization and QOL scores. It causes them to live their lives in a completely different way—to avoid normal daily-life activities for fear of yet another event

Challenge: The burden of altering daily life is not captured in QOL trial data in a way that is captivating or specific to the disease

Approach: Tell the patient stories

- Present qualitative surveys that can include patient narratives and quotes
- Include a patient speaker at a conference symposium
- Allow a patient to be an author

Providing the Road Map to the Patient Journey



The long odyssey to diagnosis can include misdiagnoses, suboptimal treatments, and unnecessary surgeries



Even after diagnosis, partnering with the right providers and selecting the correct therapy (or management strategy) can be challenging



Managing AEs and comorbidities can involve multiple specialists



Many treatments for rare diseases require a lifelong commitment

A woman with long blonde hair, wearing a light blue button-down shirt, is seated and gesturing with her hands while speaking to a man whose back is to the camera. He is wearing a grey shirt. They are in an office environment with a white brick wall and a wooden shelving unit in the background. The image is framed by a large orange triangle on the right side.

Case Study: “Staying the Course”

Situation: Patients with a rare disease may have unique challenges with adherence to therapy

Challenge: After finally getting the proper diagnosis and initiating treatment, many patients with a rare endocrine condition were discontinuing therapy for a variety of reasons

Approach: Catalog the problems to reveal the solutions

- Define the steps in the patient journey
- Highlight factors that contribute to therapy discontinuation at each step
- Develop educational strategies to support therapy maintenance

Smaller Trials Can Leave Unanswered Questions

- Phase 3 trials for rare diseases could include as few as 10 to 50 patients per treatment group
- Trial protocols and data can be a bit messier because of the need to be more inclusive of patients
 - Unforeseen issues arise more frequently because the trial designs are not established optimally yet
 - The sites may have little experience with the disease



Case Study: “Case Studies”



Situation: Mild hyperglycemia was associated with treatment of rare pituitary disease. This caused inconsistent management and reticence among endocrinologists (ironically) to use the therapy despite the efficacy advantages shown in the trial

Challenge: The primary analysis of the data only included the prevalence of AEs, with little details about disease management

Approach:

- Present a congress presentation on case studies with a subsequent manuscript
- One topic was a patient with treatment-emergent hyperglycemia that was managed
- Publish a patient population survey post study

Key Summary Points to Leveraging Patient-Level Data



Patient stories are interesting—find ways to connect to them



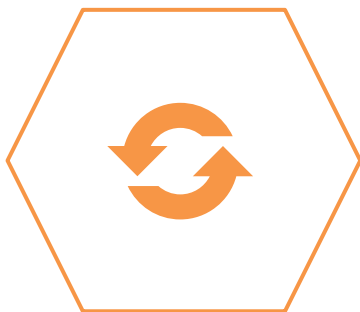
Focus on the communication objectives and determine whether patient-level data complement other approaches



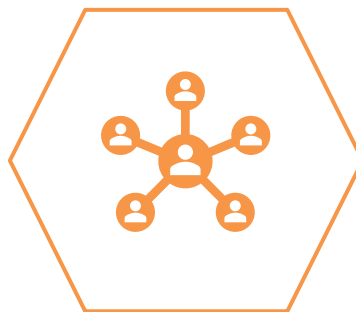
Consider case studies as an option to extend the reach of trial data

Expanding the Community

Expanding Rare Disease Expertise




Update expert guidance



Make the experts available
to the broader field



Leverage the energy of the
specialists to develop the
next generation of experts



Case Study: “Seeking Guidance”

Situation: Guidelines for disease state management were last established several years ago, and the guidance was broad and resulted in inconsistent disease management

Challenge: There may not be plans for a society to update the guidelines in a way that meets more practical needs of the physicians


Approach:

- Organize experts in a Delphi-style consensus panel
- Present consensus at conferences and in a manuscript

Providing More Access to Experts

- Applying innovative approaches can expand the reach of experts, giving less-experienced treaters more knowledge and confidence with rare diseases
- In-person disease state symposium (significant effort, moderate reach, face-to-face interaction)
- Webinars (moderate effort, broad reach, virtual interaction)





Case Study: “Train the Trainers”

Situation: The number of rare disease experts is limiting to raising awareness

Challenge: The experts are limited in their time to participate in disease state awareness initiatives

Approach:

- Organize a small group of experts to prepare simple and informative modules to present to groups of specialists
- The specialists learn the modules so that they can present the information in their institutions or regions
- As more specialists are learning more about the disease, some will also want to present this at their professional society

Key Summary Points to Expanding the Community



- Disease state awareness for rare diseases is about elevating knowledge
 - Empower generalists to refer patients to the correct specialists
 - Provide the tools for correct diagnoses among specialists
 - Ensure that expert guidance is clear and updated



- More experts means more expertise

Conclusions

Conclusions



Medical communications about rare diseases offer not only unique challenges but also exciting opportunities



It is critical to have a well-conceived communication plan that addresses the most important topics to the correct audience



Innovative tactics are often needed to raise awareness

THINK BEYOND THE ORDINARY

Thank you!

- Have a question for the panel?
Please email ismpp@ismpp.org
with the subject line: “Rare
disease session”



Acknowledgements

Special thanks to Jeff Miller, MedThink Communications, and Todd Parker, MedThink SciCom, for insight and review. Editorial assistance and graphic design support were provided by Chris Lawrence and Loftin Barnes, MedThink SciCom.

Virtual 16th Annual Meeting of ISMPP

The Evolving Role of the
Scientific Communications
Professional in an Open World



June 16-18, 2020 | Presented Virtually