THE 2019 MEDICAL AFFAIRS IN RARE DISEASES FORUM

Learn from peers and disruptive newcomers about the role Medical Affairs plays in precision medicine

dge DYNAMIC GLOBAL EVENTS

ACMA

TUESDAY & WEDNESDAY, JULY 23-24

Philadelphia, PA

PRESENTED BY DGE & ACMA

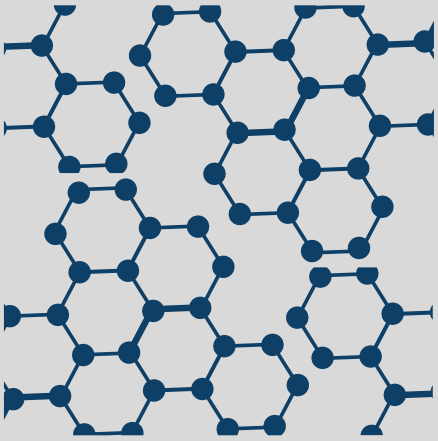
CONFERENCE OVERVIEW

The Accreditation Council for Medical Affairs and Dynamic Global Events are excited to announce a partnership that brings a CE/CME-accredited conference experience to medical affairs professionals specializing in rare diseases.

As advances in genomic and clinical science have created innovative opportunities to design personalized medicine, the pharmaceutical industry must work to reconstruct their business models to combine market opportunities with clinical health benefits. Join us at the Medical Affairs in Rare Diseases Forum to learn from peers and disruptive newcomers about the role Medical Affairs plays in precision medicine.

WHY ATTEND?

- **Earn CME/CE credits**
- **Discover how medical affairs teams are creating advanced strategies and achieving success in rare disease markets**
- **Optimize your medical affairs' ability to understand patient needs and create patient-centric medical information**
- **Understand the regulatory pathway and nuances for the orphan drug approval process and the value of natural history studies**



FEATURED SESSION TOPICS:

- Analyzing Medical Affairs' Role in Orphan Drug Development
- Working with Patient Advocacy Groups: Best Practices
- What Regulators are Looking for from Medical Affairs in Rare Diseases
- Skills & Development Training for Medical Affairs Professionals in Rare Diseases
- Assessing the Orphan Drug Market: What's Coming Next?

Seth Fritts
Global Genes

Chris Garabedian
Xontogeny

Melanie Blank
FDA

Jaime Arvizu
Ultragenyx

Nadia Bodkin
Blaze Therapeutics

Patient Engagement Strategy Workshop
Monday, July 22, 2019

11:30 – 12:30
Registration & Networking Lunch

12:30 - 1:00
Opening Interactive Exercises
· Define your objectives and what you hope to achieve
Terri Ellsworth, *Director Professional Relations, Patient Advocacy*, **Accreditation Council for Medical Affairs (ACMA)**

Meredith Cagle, *Senior Director, Patient Engagement,* **Global Genes**

1:00 - 2:00
PART 1: Patient Advocacy in Recent News and What It Means to Medical Affairs
· Patient Advocates of the Future & Evolving Trends
· Discuss and understand how to adopt a patient engagement strategy at your company
· Distinguish tactics to make patient engagement during the drug development lifecycle a permanent practice

2:00 - 2:45
PART 2: Designing Protocol with Advocates
· Discuss how to compose protocols that involve patients at the beginning of drug development
· Learn to build an experience that considers the needs of the patient
· Examine the changing role of patient advocacy and review how to empower patient advocates
Helena Chung, *Global Patient Reported Outcomes Scientist for Oncology,* **AstraZeneca**

2:45 - 3:00
Networking & Refreshment Break

3:00 - 3:45
PART 2: Designing Protocol with Advocates *(continued)*
· Review how to continually engage with patients throughout the trial process
· Address rare diseases and how to satisfy the unmet needs of patients and providers
· Implement strategies to overcome limitations that patients and providers encounter in rare and complex diseases
Helena Chung, *Global Patient Reported Outcomes Scientist for Oncology*, **AstraZeneca**

3:45 - 4:30
PART 3: Improving Patient Materials
· **Understanding data**
· **How to provide accurate patient-friendly information**
· **Creating metrics**

4:30 - 5:00
PART 4: Technology Solutions That Help to Empower the Patient & Improve Patient Engagement

5:00 - 5:30
Reflection & Action Plans

Caryl Harris, *Patient Ambassador*, **Illumina**
Founder & Executive Director, **Avery's Hope**

Nina Daya
Rare Disease Parent and Patient Advocate

Terri Ellsworth, *Director Professional Relations, Patient Advocacy,* **Accreditation Council for Medical Affairs (ACMA)**

Medical Affairs in Rare Diseases Day One:
Tuesday, July 23, 2019

8:00– 9:30
Registration & Continental Breakfast

8:30 AM – 9:30 AM
Women's Leadership in Medical Affairs Breakfast
(optional)
Join us for a morning of inspiration and support as the ACMA and DGE present the first annual Women's Leadership in Medical Affairs Breakfast. Engage in the conversation as panelists discuss what helped drive and develop them to become the leaders they are today.

- Explore the challenges facing women leaders in pharma
- Discuss the critical points in a woman's career where advancement is possible
- Create a road map for achieving your next leadership role in Medical Affairs
- Discuss strategies for fostering diversity and inclusion

Meg Heim
Vice President, Head of NA Medical Affairs Scientific Communications, CV and Diabetes
Sanofi

9:30 – 9:45
Chairperson's Opening Remarks

Jaime Arvizu
Vice President, Head Medical Affairs, LATAM
Ultragenyx

9:45 – 10:30
Keynote Address: Medical Affairs' Role in Rare Disease Drug Development
As accessibility to medical information has exploded over the last 20 years and moved online, it has changed the way that researchers, clinicians and patients receive information and has had an important influence in the collective decision-making process in what treatment a patient will receive. The most important function across the biopharmaceutical industry in facilitating accurate and balanced information about the treatment choices available is the Medical Affairs function. This is even more evident in the area of Orphan Drugs and Rare Disease where there are often limited FDA-Approved treatment options, an emergence of new investigational drugs and genetic technologies, and often a greater engagement among patients, patient advocacy organizations and disease foundations.

- How to employ the optimal Medical Affairs strategy to serve effectively in this complex new environment in serving the research, clinical and patient communities with the information they need
- How to meet the increasing demand for information from medical conferences on a real-time basis for both FDA-approved drugs and investigational drugs in development
- How to use the Medical Affairs function to guide drug development and increase the probability of success for regulatory approval and to create best-in-class therapies for patients

Chris Garabedian
Chairman and CEO, **Xontogeny**
Former CEO, **Sarepta Therapeutics**

10:30 – 11:15

Working with Patient Advocacy Groups: Best Practices

In this session, we'll learn how to optimize medical affairs' ability to understand patient needs and create patient-centric medical information. We will explore methods for working with patient advocacy groups to engage patients, understanding their needs, and encouraging patient-centered views and developments.

- Understand what patient advocacy groups want from medical affairs
- Describe best practices for working with patient advocates
- Learn about where the patient advocacy space is heading in the next decade

Jodie Sherman Gillon, *Global Medical Lead, Patient Engagement Rare Diseases,* **Pfizer**

Susan Walther, MS, CGC, *Director of Patient Engagement*
Friedreich's Ataxia Research Alliance (FARA)

Seth Fritts, *Corporate Engagement*
Global Genes

Terri Ellsworth, *Director Professional Relations, Patient Advocacy*
Accreditation Council for Medical Affairs (ACMA)

11:15 – 11:30

Networking & Refreshment Break

11:30 – 12:15

Creating an Expedited Pathway for Industry to Collaborate with Patients

Discuss how advocacy groups can be leveraged to create standards that help the industry understand rare diseases and gain the ability to develop drugs more successfully for patients in need.

Nadia Bodkin, *CEO,* **Blaze Therapeutics**

12:15 – 1:15

Networking Lunch

1:15 – 2:00

What Regulators Look for from Medical Affairs in Rare Diseases

There have been notable regulatory actions taken in the rare disease space. However, specific guidance for orphan drug development is still limited compared to traditional drug development. Discuss what tools the FDA offers to promote and advance orphan drug licensing and how to work with regulators throughout the lifecycle of the drug.

- Learn about how to effectively engage with regulators focusing on rare diseases
- Discover the value of natural history studies and their impact on the regulatory pathway
- Discuss the differences between FDA vs EMA and other regulatory agencies on orphan drug approval

Melanie Blank, *Medical Officer,* **FDA**

2:00 – 2:45

Set-Up Your Organization for Success: Medical Affairs Departmental Structure in Rare Disease Organizations

Examine the impact that rare disease has on medical affairs departments and consider the best approach for developing an organizational strategy that aligns with the changing standard in the pharmaceutical industry to personalized medicine.

- Examine successful organizational models for medical affairs in rare diseases
- Learn the impact of key roles such as patient diagnosis, patient diagnostics and medical affairs shared services
- Compare & contrast biotech vs. small to mid-size vs. large pharma organizations in rare diseases

Jaime Arvizu, MD, MPH, *Vice President, Head Medical Affairs, LATAM,* **Ultragenyx**
Dr. Anne Arvizu PharmD, FASCP, PCC, *CEO and Medical Affairs Advisor,* ***RXER Communications***
Marissa Ricci, *VP Patient Diagnosis Program*, **Ultragenyx**

2:45 PM – 3:00 PM

Networking & Refreshment Break

3:00 – 3:45

Explore the Intersection of Rare Disease and Gene Therapy

Paul Korner, M.D., M.B.A., *SVP, Clinical Development & Medical Affairs,* **Axovant Sciences**

3:45 – 4:30

How Does One Find Key Opinion Leaders for a Rare Disease?

As pipelines continue to shift focus to rare diseases, it is crucial to understand and engage thought leaders to support scientific exchange. Medical affairs teams will need to have a more organized strategy when planning for KOL engagements.

- Identify Key Opinion Leaders and Thought Leader Engagement is a core responsibly for Medical Affairs teams
- Discuss the significant differences in how this should be done for rare diseases compared to other larger more well-known diseases
- During this session examine what those differences are and how to optimize the process for the rare disease market

Ariel Katz, *Chief Product Officer,* **H1 INSIGHTS**
Ann Leon, *Director, Immunology Medical Therapeutic Area***, CSL Behring**

4:30 – 4:45

Day One Closing Remarks
Jaime Arvizu, *Vice President, Head Medical Affairs, LATAM***, Ultragenyx**

4:45

Day One Concludes

Medical Affairs in Rare Diseases Day Two:
Wednesday, July 24, 2019

8:00 – 9:00
Networking & Breakfast

9:00 – 9:15
Chairperson's Recap of Day One
Jaime Arvizu, *Vice President, Head Medical Affairs, LATAM*, **Ultragenyx**

9:15 – 10:00
Clinical Trial Design Challenges in Rare Diseases
Creating patient-focused clinical trials with genetic or precision medicine will help to create stronger targeted therapies for patients in need. Cross-functional collaboration is crucial to assure the perspective and expertise of the patient population are included in clinical development.

- Describe the challenges facing clinical development in rare diseases
- Outline biostatistical methods that can be used to study rare diseases with a heterogeneous phenotype
- Provide case studies of innovative clinical trial designs for successful orphan drug launches
- Adding genetic testing and gene-based stratification into clinical trial design

10:00 – 10:45
Medical Affairs Touchpoints Across the Orphan Drug Lifecycle
Develop clear processes and methods to collect and integrate insights from different stakeholders. Leverage these insights by collaborating with R&D and commercial to increase productivity and expedite orphan drug approval and commercialization.

- Learn about how medical affairs can work effectively with clinical, regulatory and commercial to prepare for a successful drug launch
- Understand the role of laboratory and diagnostic services and their role in the proper identification of patients
- Learn about how medical affairs can be a strategic driver in setting your organization up for success

Uzma Atif PhD, MPH
Senior Medical Science Liaison, US Rare Disease (LSD)
US Medical Affairs, Global R&D
Takeda

Ekaterina Wright
Global Medical Lead Rare Diseases, Medical Affairs
Takeda

10:45- 11:15
Rare Is Rare: How Many Zebras Are There, and Where Are They Hiding in the Medical Literature?
Rare diseases are an integral part of the medical landscape. By the very nature of their limited prevalence, very little is known about the majority of rare diseases. Consequently, whatever is known needs to be of high quality, easily found, and freely accessible to healthcare providers to enable them to manage the disease in their patients. Just as important, quality information needs to be freely accessible to patients, advocates, and caregivers (PACs) so that they can be well informed and participate fully in their treatment regimens.

- Understand the prevalence of rare communications in the medical literature database
- Explore where rare manuscripts are published
- Take a deeper dive into who has access to the articles, article type, and the acknowledged involvement of PACs, including as authors.

Dan Donovan, *Co-Founder & CEO,* **rareLife solutions**

11:15 – 11:30
Networking & Refreshment Break

11:30 – 12:30
Maximizing Field Medical Teams in Rare Disease
MSLs looking to generate true influence will need the right training and technological support. Explore different avenues and technologies that allow MSL teams to excel when working with the scientific sophistication of today's pharmaceutical products, particularly genomics and orphan drugs.

- Learn about the skills required to work in rare diseases and how to develop your team accordingly
- Understand the evolution of field medical and where to focus resources for maximum ROI
- Learn how to make the case for increased investment in field medical affairs and greater influence with internal and external orphan drugs stakeholders

12:30 – 1:30
Networking Lunch

1:30 – 2:15
Skills & Development Training for Medical Affairs Professionals in Rare Diseases
Training medical affairs specialists in rare diseases can be challenging. Finding and developing leaders that can be leveraged for sustained success will ensure your organization will be successfully operating in rare diseases.

- Learn about the necessary skills needed to prepare your medical affairs organization to be highly functioning in rare diseases
- Discuss the core areas of training needed for medical affairs professionals working in rare diseases
- Understand the value of broad skills training for medical affairs professionals in rare diseases

2:15 – 2:30
Networking & Refreshment Break

2:30 – 3:15
Orphan Drug Market: What's coming?
There are 7,000 known rare diseases but only a few hundred have federally approved treatments. Orphan drug manufacturers face unique obstacles to market. Examine new ways to drive the development of medicines to treat rare diseases.

- Learn about the latest developments in the orphan drug market
- Explore effective partnerships with external rare disease organizations
- Understand the latest trends in healthcare policy related to rare diseases

3:15 – 3:30
Chairperson's Closing Remarks
Jaime Arvizu, *Vice President, Head Medical Affairs, LATAM,* **Ultragenyx**

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