CLINICAL TRIALS IN RARE DISEASES

2023

13-14 September 2023

Princeton, **New Jersey**

SPEAKERS

Gabrielle DeBartolomeo, Clinical Trial Scientist, **Alkeus Pharmaceuticals**

Daniel Klamer, PhD, MBA, Vice President, Business Development and Scientific Strategy, **Anavex Life Sciences Corp**

Chris Adams, Chief Executive Officer, **Andarix**

Jeff R. Swarz Ph.D, Chief Executive Officer, **ATED Therapeutics**

Jay Russak, Senior Director, Clinical Operations, **Keros Therapeutics**

Keith Chiasson, Vice President, Drug Development, **Feldan Therapeutics**

Laurie Taraborrelli, Clinical Programs and Compliance Specialist, **Fujirebio Diagnostics**

Julie Breneiser, Executive Director, **Gorlin Syndrome Alliance**

Karin Hoelzer, DVM, PhD, Director, Policy and Regulatory Affairs, **National Organization for Rare Disorders (NORD)**

Michele Rhee, Rare Disease Patient and Head of Global Advocacy Relations and Patient Engagement**, Savara Inc**

Ravipal Luthra, Clinical Research Lead, **Sylvester Comprehensive Cancer Center, University of Miami**

Karen L. King, Vice President, Clinical Operations, **Recursion Pharma**

Noopur Singh, Director, Medical Affairs, **Xentria**

CLINICAL TRIALS IN RARE DISEASES

13TH-14TH SEPTEMBER | PRINCETON, NJ

Clinical Trials in Rare Diseases DAY 1 – Wednesday 13th September	
8:00am	**Registration and refreshments**
8:50am	**Chairperson's opening remarks** **Chris Adams,** Chief Executive Officer, **Andarix**
9:00am	**OPENING KEYNOTE: Participating in a clinical trial from a patient perspective: how can biotech and pharma sponsor companies reduce the burden on patients?** • How it feels to participate in a clinical trial as a patient and an overview of the main barriers to patients • Understanding the financial impact of trial participation on patients: where can sponsors help? • Decentralization as a solution to ease burden on patients and improve access to trials, particularly for rare diseases • The role of rare disease study sponsors in supporting people diagnosed with rare diseases **Michele Rhee,** Rare Disease Patient and Head of Global Advocacy Relations and Patient Engagement, **Savara Pharmaceuticals**
9:30am	**Session reserved for Premier Research**
10:00am	**PANEL DISCUSSION: How decentralization and direct-to-patient shipping opens up possibilities for patients with rare diseases** • Improving enrolment, retention and access to clinical trials through decentralized and hybrid clinical trial models • Incorporating direct-to-patient shipping in order to make participating trials easier for patients and challenges associated with DTP • DCT and DTP as a way to open up trial participation to pools of patients who would otherwise have been unable to access trials • Assessing home nursing and hybrid options for when full decentralization is not possible due to the nature of your drug **PANELLISTS:** **Keith Chiasson,** Vice President, Drug Development, **Feldan Therapeutics**
10:30am	**Morning refreshments and networking**
11:15am	**Improving ROI by working with true disease experts: patients, and how working with advocacy groups can impact trial success** • Methods used to collaborate with rare disease advocacy groups from trial design forward • Working with advocacy groups to understand challenges and barriers patients face, and how you can support participants throughout the clinical trial in order to increase retention rates and improve experiences • Looking at alternative endpoints in rare disease **Julie Breneiser,** Executive Director, **Gorlin Syndrome Alliance**

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11:45am	**Session reserved for Novotech**
12:15pm	**An update on regulations and policy surrounding rare disease and orphan drug trials: what do you need to know?** • FDA, NIH and CMS issues impacting rare disease and orphan drug trials • Utilizing real world evidence effectively and key considerations when using this kind of data • Understanding regulatory flexibility for clinical trial diversity and decentralization in orphan drug trials **Karin Hoelzer, DVM, PhD,** Director, Policy and Regulatory Affairs, **National Organization for Rare Disorders (NORD)**
12:45pm	**Lunch and networking**
2:00pm	**How can you improve diversity and inclusion in rare disease trials?** • FDA guidance and legislation around diversity in clinical trials and how to apply this in a rare disease trial • How to increase awareness of your trial in order to reach more patients including those in more diverse settings • The importance of building trust with groups and communities who may not traditionally participate in clinical trials in order to increase trial diversity and ultimately improve the effectiveness of your drug **Laurie Taraborrelli,** Clinical Programs and Compliance Specialist, **Fujirebio Diagnostics**
2:30pm	**Session available for sponsor**
3:00pm	**New solutions and tactics for enrolling patients in rare disease trials** • Finding patients who are 'off the grid': how can you identify potential trial participants who have given up on finding a treatment for their rare disease? • Leveraging the power of social media in your patient enrolment campaign: using tools such as Facebook groups and other platforms in order to reach out to higher numbers of patients • What new technology is available to support patient recruitment and how can you employ this effectively to reduce timelines for enrolment, and therefore the overall length of your trial? **Jay Russak,** Senior Director, Clinical Operations, **Keros Therapeutics**
3:30pm	**Afternoon refreshments and networking**
4:15pm	**CASE STUDY: Working with a patient advocacy group to drive recruitment for a rare disease trial** • Best practice in engaging patient advocacy groups early on in the clinical trial process in order to ensure your trials are as patient-friendly as possible • Collaborating with advocacy groups in order to find potential patient participants and encourage trial participation • The benefits of working closely with patients, patient advocates and advocacy groups in order to ensure your trial is a success **Karen L. King,** Vice President, Clinical Operations, **Recursion Pharma**
4:45pm	**Session available for sponsor**

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5:15pm	**CASE STUDY: Clinical trials for rare diseases: challenges and opportunities** • Best practice in ensuring your rare disease trial is a success • Using precision medicine in clinical trials to advancing therapeutics for rare diseases • Assessing clinical data with biomarker-correlated outcomes in Rett Syndrome **Daniel Klamer, PhD, MBA,** Vice President, Business Development and Scientific Strategy, **Anavex Life Sciences Corp**
5:45pm	**Chairperson's closing remarks** **Chris Adams,** Chief Executive Officer, **Andarix**

END OF DAY 1 AND NETWORKING DRINKS

CLINICAL TRIALS IN RARE DISEASES

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Clinical Trials in Rare Diseases DAY 2 – Thursday 14th September	
8:00am	**Registration and refreshments**
8:50am	**Chairperson's opening remarks** **Chris Adams,** Chief Executive Officer, **Andarix**
9:00am	**PROBLEM-SOLVING ROUNDTABLE DISCUSSIONS** *During the roundtable discussion session, the conference hall will be divided into four 'zones'. Delegates can choose which zone they would like to join. Each zone will be led by a table moderator and will focus on a different challenge within rare disease and orphan drug clinical trials. After 45 minutes, delegates will have the opportunity to swap and choose a different table, and each roundtable will run twice.*
	ROUNDTABLE 1: Considerations when designing a clinical trial for a rare disease: challenges and solutions **Jeff R. Swarz Ph.D,** Chief Executive Officer, **ATED Therapeutics**
	ROUNDTABLE 2: Creating a patient friendly clinical trial: working with advocacy groups effectively **Julie Breneiser,** Executive Director, **Gorlin Syndrome Alliance**
	ROUNDTABLE 3: Identifying partners and vendors to maximize efficiency of your rare disease trial **Laurie Taraborrelli,** Clinical Programs and Compliance Specialist, **Fujirebio Diagnostics**
10:30am	**Morning refreshments and networking**
11:15am	**Engaging patients beyond just participating in a trial: the role of biopharma companies in supporting patients** • Where rare disease trial sponsors can step in to fill scientific advice gap and teach patients about their condition • The importance of making patients feel cared for throughout and beyond trial participation • How rare disease trials differ from other clinical trials when it comes to supporting patients in understanding and managing their disease long term **Gabrielle DeBartolomeo,** Clinical Trial Scientist, **Alkeus Pharmaceuticals**
11:45am	**Session available for sponsor**
12:15pm	**CASE STUDY: A site perspective on running a clinical trial for a rare disease** • Overcoming challenges involved when in a trial with an aggressive disease • Learnings and outcome of running clinical trials on amyloid leukemia and glioblastoma tumors • How sponsors can best work with sites effectively to ensure rare disease trials run as smoothly as possible **Ravipal Luthra,** Clinical Research Lead, **Sylvester Comprehensive Cancer Center, University of Miami**
12:45pm	**Lunch, networking and prize draw!**

CLINICAL TRIALS IN RARE DISEASES

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1:45pm	**CASE STUDY: From bench to bedside: taking an orphan drug from the lab to a clinic** **Noopur Singh,** Director, Medical Affairs, **Xentria**
2:15pm	**Session available for sponsor**
2:45pm	**CLOSING KEYNOTE PANEL: Looking to 2024 and beyond: what's on the horizon for rare disease and orphan drug trials?** • With this year as the 40th anniversary of the Orphan Drug Act, how have clinical trials evolved over that period? • New regulations and guidance for running orphan drug trials in the USA: how will these impact trials? • Are we still seeing long term changes as a result of COVID? How the pandemic made trials become more flexible: are these changes here to stay? • Developments in patient recruitment: leveraging technology to shorten timelines and achieve as diverse a patient population as possible • Should decentralized trials be the norm, particularly when working with rare diseases? **PANELLISTS:** **Jay Russak,** Senior Director, Clinical Operations, **Keros Therapeutics**
3:15pm	**Chairperson's closing remarks** **Chris Adams,** Chief Executive Officer, **Andarix**
END OF CONFERENCE	

Additional topic suggestions:

CASE STUDY: Designing and running a trial for rare pediatric cancers: overcoming challenges when working with younger patients

- Working with and supporting caregivers throughout the clinical trial process, and where sponsors can relieve burden
- Additional considerations when designing a pediatric study: best practice when working with children and teenagers
- Regulatory guidance in the USA around enrolling under 18s in a clinical trial and how to navigate this

FDA regulations around orphan drug designation and expedited approval: what you need to know

- Navigating hurdles around data and evidence for studies with smaller numbers of patients
- The benefits of FDA Orphan Drug Status in the US and how this can help make your study more efficient
- Ensuring early access to drugs and how to make sure drugs are both safe and quickly available to the American market

With sites facing high levels of staff turnover and high vacancy numbers, how can sponsors reduce the burden on sites?

- Mitigating problems created by high levels of staff turnover at sites: where can sponsor companies make processes easier to alleviate workload of site staff?
- Supporting and training staff throughout your clinical trial, from patient enrolment to data entry, in order to shorten overall timelines
- The importance of building and maintaining a strong relationship with your trial site and how this can be make or break for your study

Choosing a CRO for your rare disease study: factors to consider

- Is therapeutic knowledge always key when selecting a CRO for a rare disease study?
- Weighing up the benefits of smaller specialized CROs vs larger global CROs
- Additional challenges to the CRO selection process when dealing with ultra-rare diseases where CROs may have little to no experience of the illness
- Debating the benefits and challenges of employing a full service CRO for your rare disease trial vs working with multiple vendors throughout your study