Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC) Adjunct Scientific Workshop

Workshop held: July 20, 2023, 12:00 – 2:00 PM ET

Report date: November 7, 2023

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Executive Summary

The Sudden Arrhythmia Death Syndromes (SADS) Foundation exists to save the lives and support the families of children and young adults who are genetically predisposed to sudden death due to heart rhythm abnormalities. Arrhythmogenic right ventricular cardiomyopathy (ARVC) is a rare, genetically determined progressive condition. ARVC results in a progressive replacement of right ventricular myocardium with fatty and fibrous tissue, leading to the weakening of the right ventricle, resulting in irregular heartbeats or ventricular arrhythmias. Half of patients develop heart failure symptoms, and some require a heart transplant.

The SADS Foundation hosted an ARVC Adjunct Scientific Workshop on July 20, 2023. ARVC clinical and research experts reflected on ARVC patient perspectives that they had heard during an externally led patient focused drug development (EL-PFDD) meeting on June 20, 2023. They discussed ways to best ensure that therapy development and clinical trials can best support the needs of the ARVC community.

Expert Discussion Points:

1. Recognize and acknowledge that ARVC patients are at risk of a serious mental health burden from this disease.
2. Reducing arrhythmia burden will have a profound and dramatic effect on reducing anxiety and depression.
3. Develop methods and measures to provide a more comprehensive view of ARVC including better ways to identify and quantitate the persistent stresses and mental health impacts of this disease.
4. Clinical trial endpoints must (1) be aligned with the things most important to those living with ARVC; and (2) demonstrate clinically meaningful change.
5. Selection criteria for future gene therapy needs to be carefully determined.
6. Anxiety levels of patients in future gene therapy trials will need to be tested and monitored, and appropriate support provided.
7. Although research into ARVC gene therapy is promising, realistic expectations about timelines, treatment opportunities and outcomes are necessary.
8. Gene therapy is not yet a consideration for asymptomatic ARVC patients.

The ARVC patient community hopes to have better treatments become available for themselves and their affected family members as a result of this discussion.

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Patient Focused Drug Development for ARVC

Arrhythmogenic right ventricular cardiomyopathy (ARVC)

Arrhythmogenic right ventricular cardiomyopathy (ARVC) is a rare, genetically determined disease with serious consequences. ARVC results in a progressive replacement of right ventricular myocardium with fatty and fibrous tissue, leading to the weakening of the right ventricle, resulting in irregular heartbeats or ventricular arrhythmias.

Two-thirds of ARVC patients have a genetic variant affecting a desmosome protein including plakophilin-2 (*PKP2*), desmoplakin (*DSP*), plakoglobin (*JUP*), desmoglein-2 (*DSG2*), or desmocollin (*DSC2)*. Other non-desmosome related genes can also be involved in this disease.

Exercise exacerbates the disease in those at risk for developing ARVC; the identification of a genetic variant in a young person is a strong enough predictor of the disease that these individuals are recommended to prophylactically avoid competitive and endurance sports.

ARVC typically presents around the age of 29, with palpitations, syncope (fainting), sudden death, or resuscitated sudden death. ARVC is progressive, with a gradual decline of right and left ventricular function and right ventricular dilation. Heart failure is a late manifestation of ARVC, usually occurring 10 to 20 years or more after the first symptoms develop. ARVC has an enormous unmet medical need. Half of patients develop heart failure symptoms, and some require a heart transplant.

Patient Focused Drug Development

The United States Food and Drug Administration (FDA) first launched the patient focused drug development initiative in 2012. This program recognizes that patients living with a disease not only have a direct stake in the outcome of FDA decisions but are in a unique position to contribute to the understanding of their disease.

The FDA is interested in two particular categories of the patient experience: (1) **Analysis of Condition** includes the burden of disease and impacts on activities of daily living; (2) **Current Treatment Options** includes patients' perspectives on the adequacy of available therapies to help understand unmet medical needs and prospects for future treatments. This information is considered within a structured risk-benefit decision making framework, and helps the FDA calibrate exactly how much risk a patient population will tolerate for a given set of benefits. Additional resources are listed in **Appendix 1**.

The *Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC) Externally-Led Patient-Focused Drug Development (EL-PFDD) Meeting* was held on June 20, 2023. During this meeting, individuals living with ARVC shared their experiences with the FDA, clinicians, researchers, industry, and advocacy organizations to help them to understand the profound burden of ARVC and the urgent need for better treatments.

Key information from the June 20, 2023 ARVC EL-PFDD meeting are presented in a ~~benefit-~~risk-benefit framework as a foundation for the FDA's Benefit-Risk Assessment (see Table 1). Note that the information in this sample framework is likely to evolve over time.

Table 1: ARVC Benefit-Risk Table Developed at the June 20, 2023 ARVC EL-PFDD Meeting

	EVIDENCE AND UNCERTAINTIES	CONCLUSIONS AND REASONS
ANALYSIS OF CONDITION/ IMPACTS ON ACTIVITIES OF DAILY LIVING	**Arrhythmogenic right ventricular cardiomyopathy (ARVC) is a rare, progressive genetic disease, that can lead to heart failure and death**. Diagnosis is often unexpected and multiple family members can be affected. **The most burdensome ARVC-related health effects include arrhythmias/ palpitations, fatigue, anxiety/ depression**, **exercise intolerance, sudden cardiac arrest, and heart failure.** Many live with a combination of these symptoms. Symptoms are interconnected; arrhythmias, palpitations and shocks cause anxiety and PTSD, which can result in more arrhythmias, palpitations, and shocks. ARVC is progressive and 49% of those living with ARVC develop heart failure.	**ARVC families have many worries.** They worry about other family members, especially younger adults, teens, and children who have inherited an ARVC gene variant and do not yet show symptoms. They worry about progression to heart failure, death, and sudden cardiac arrest. They worry about arrhythmias and palpitations leading to more shocks**.** **ARVC has broad impacts.** Exercise promotes progression, so exercise and participation in sports is restricted. This worsens anxiety and depression and forces many affected with ARVC to reconsider their self-identities and family roles, further contributing to the tremendous mental health burden of this disease.
CURRENT TREATMENT OPTIONS/ PROSPECTS FOR FUTURE TREATMENTS	**Despite the potentially life-threatening manifestations and daily symptoms of ARVC, there are no FDA-approved treatments that are curative or stop progression.** Individuals living with ARVC require multiple medical therapies to manage their disease, including implantable cardioverter-defibrillators (ICD) and combinations of medications. Most also use exercise moderation and lifestyle modifications to manage symptoms. **Treatments and lifestyle choices improved their quality of life but are accompanied by significant downsides.** ICDs cause sudden unexpected shocks, leading to major depression, anxiety, and PTSD. Side effects of beta blockers and antiarrhythmic medications impact quality of life. Some patients require multiple cardiac ablations. Some will eventually require a heart transplant.	**Individuals living with ARVC need a medication to stop advanced heart failure, arrhythmias/palpitations, and the risk of sudden cardiac arrest.** Although even minor medication improvements would improve their quality of life, they want a disease modifying therapy such as gene therapy. Heartbreakingly, many living with ARVC recognize that they have progressed too far for a disease modifying treatment to help them but wish for a treatment to help others, especially for those who are still asymptomatic. They also need improved/enhanced ICD/devices, less medication side effects, more ARVC education, awareness and research, more mental health support.
	See the Voice of the Patient report for a more detailed narrative.	

Adjunct Scientific Workshop Summary

The ARVC Adjunct Scientific Workshop, held on July 20, 2023, was a critical follow up to the EL-PFDD meeting. ARVC clinical and research experts reflected on ARVC community input, and discussed how to ensure that therapy development and clinical trials can best support the needs of the ARVC community. The meeting was cohosted by **James Valentine**, JD, MHS and **Genevie Echols**, RCIS, Family Support Director for the SADS Foundation. The Adjunct ARVC Scientific The workshop agenda is in **Appendix 2**.

Genevie Echols provided opening remarks and reviewed the meeting structure. **Larry Bauer**, RN, MA, Senior Regulatory Drug Expert, Hyman Phelps, & MacNamara, P.C., summarized the key points from the ARVC EL-PFDD meeting, highlighting the most significant ARVC symptoms and impacts and top unmet medical needs identified by patients. **James Valentine** explained a general framework for integrating the patient voice in drug development, as a foundation for the rest of the meeting.

Physician scientist, **Dr. Hugh Calkins,** MD, Director of the ARVC/D program at Johns Hopkins University, spoke about opportunities for patient-centered treatment approaches in ARVC. **Dr. Sam Sears,** PhD, ABPP from the Departments of Psychology and Cardiovascular Sciences at East Carolina University, provided a landscape analysis of tools to evaluate what's important to ARVC patients.

Experts participated in a panel discussion to integrate the perspectives of the ARVC community and to discuss ideas to help facilitate therapeutic development and influence clinical trial design. In addition to Dr. Calkins and Dr. Sears, the discussion included **Dr. Mario Delmar**, MD, PhD, a professor of cardiology at NYU Langone, **Dr. Wojciech Zareba**, MD, PhD with the Cardiovascular Clinical Research Center at the University of Rochester Medical Center, and **Brittney Murray**, MS, CGC, Genetic Counselor with the Johns Hopkins ARVD/C program.

Dr. Michael Ackerman, MD, PhD, President of the SADS Foundation Board of Trustees, and a genetic cardiologist at the Mayo Clinic, summarized the expert discussions and concluded,

> "*Live and thrive. We've got to start working on thriving. We're not there. Status quo is not acceptable. And now's the time for all of us to do this together*."

Video recordings of the July 20, 2023 ARVC Adjunct Scientific Meeting and the June 20, 2023 ARVC Externally-Led Patient Focused Drug Development Meeting, as well as the ARVC Adjunct Scientific Workshop Report and the ARVC Voice of the Patient report are available at https://sads.org.

Adjunct Scientific Workshop Discussion and Expert Recommendations

Voice of the ARVC Community

ARVC causes a tremendous mental health burden on patients as well as their families.

At the EL-PFDD meeting, patients described how high levels of anxiety and depression are one of the most burdensome ARVC-related health effects. Anxiety and depression are distal to the disease process itself, and result from a number of different factors. These factors can include **disease-related factors**, such as the fear of heart failure and sudden death. Arrhythmias and premature ventricular contractions (PVCs) trigger anxiety, which in a vicious cycle, triggers more PVCs and arrhythmias, and can lower longer-term survival. **Treatment-related factors** also contribute to anxiety and depression. Many patients described how implantable cardioverter-defibrillators (ICD) cause sudden unexpected shocks, contributing to anxiety and PTSD. Exercise restrictions worsen anxiety and depression, and force many affected with ARVC to reconsider their self-identities and their roles within the family, further adding to the tremendous mental health burden of this disease. Complex **psychosocial factors** include fears and uncertainty about the future, social isolation, hypervigilance, and familial worry.

Expert Discussion Point 1

Recognize and acknowledge that ARVC patients are at risk of a serious mental health burden.

Experts agreed that ARVC is a disease that goes beyond pathophysiology or biologic outcomes. The symptom burden associated with PVCs and other arrhythmic events imposes both physical and mental burdens on patients. Experts also acknowledged that the enormous mental health toll that anxiety and depression extracts from ARVC patients is vastly underappreciated in the medical community. They cited evidence demonstrating due to the many ambiguities of this disease, a third of individuals living with ARVC are living with clinically significant psychological distress.

Expert Discussion Point 2

Reducing arrhythmia burden will have a profound and dramatic effect on reducing anxiety and depression.

Medically treating and reducing PVCs may be an important holistic approach in terms of treating the overall impact of this condition and supporting patients in their day-to-day functioning.

Expert Discussion Point 3

Develop methods and measures that provide a more comprehensive view of ARVC, including better ways to identify and quantitate the persistent stresses and mental health impacts of this disease.

- Having robust protocols and metrics to appropriately measure quality of life will allow clinicians to screen ARVC patients for mental health burden at baseline and during the course of their disease. Acknowledging and monitoring of patient mental health in clinical practice and during clinical trials is essential for addressing the needs of the "whole person". Patients in distress need to be provided with appropriate interventions and integrated support, not only for themselves, but for their entire family.
- Dr. Sears introduced many different models which consider a full view of health, along with metrics that could potentially be adapted for ARVC. During the panel discussion he recommended a four-point measurement system that includes: (1) **physical burden of ARVC**, which is consistent with electrophysiology, including measurement of PVC and arrhythmias; (2) **mental burden of ARVC**, which is consistent with psychology and could measure multifaceted anxiety (fear, hypervigilance, avoidance of activity, attention to symptoms, device-specific anxiety); (3) **daily ARVC impacts**, which reflect patient quality of life such as the amount of movement, ability to attend work, to care for self and to function in the family; (4) **future burden of ARVC**, related the concept of empowerment and the growth mindset, receptivity to change and openness to adaptation. Regarding future burden: what people believe about the future is a significant predictor of their present mental health.
- Dr. Sears recommended going beyond just considering and measuring quality of life, but to identify and define factors associated with adaptation, recovery, and resilience which determine whether individuals thrive or suffer.

Expert Discussion Point 4

Clinical trial endpoints must (1) be aligned with what is most important to those living with ARVC; and (2) demonstrate clinically meaningful change.

- Experts discussed ideas for identifying what is most important for patients and to find ways of measuring and monitoring these things more accurately. These may include patient-reported outcomes and patient-reported quality of life measures. Patient reported data needs to be specific, with a range of responses as opposed to open ended questions. Experts emphasized that there is a fine balance between obtaining robust data and imposing too many questions on patients. They also recommended collecting both subjective (patient or family reported) measures as well as objective measures such as activity data from wearable devices or the number of days missed from work (for example).
- Experts agreed that ARVC trials need to measure premature ventricular contractions (PVCs) as a primary endpoint. PVCs are a common cause of symptoms such as anxiety and are related to risks of arrhythmias and sustained ventricular tachycardia (VT) or ventricular fibrillation (VF) episodes. Many of these other symptoms (including VT and cardiac arrest)

occur more infrequently, so it may be challenging to demonstrate a statistically significant reduction in events in this rare disease context.

- Experts discussed the idea of objectively measuring PVC and arrhythmia burden using Zio patches or other ECG devices including ICDs, especially over a longer time frames (weeks or months). Constant monitoring is particularly important during the early stages of a given therapy, where safety is a consideration. Experts discussed potentially interrogating ICDs remotely, to reduce hospital/physician's office visits. They also discussed how wearable devices (and ICDs), equipped with accelerometers, could potentially be used to measure daily activity of patients to complement the data from patient questionnaires. Currently, different ICD manufacturers each use different programs, settings, and interpretations, making it hard to collect standardized data for comparison.
- A clinical trial challenge for ARVC is ensuring that selected endpoints eventually translate into a reduction in heart failure and a reduction in mortality. Experts commented that larger and longer duration trials may be needed to collect this data.
- Although anxiety is a significant ARVC health burden, it is not an appropriate endpoint for clinical trials. Instead, endpoints need to focus on things more proximal to ARVC but drive anxiety (for example arrythmias, shocks).
- In response to an audience question, experts stated that although there is no measure currently being developed related to novel therapies, PROs will need to be developed to accompany new medicines and therapies. In the meantime, stand in measures including shock, anxiety, ICD acceptance, and specific disease beliefs can potentially be used.

Voice of the ARVC Community

Current ARVC therapies are insufficient as they do not stop progression. Treatments and lifestyle choices can improve patient quality of life somewhat, but are accompanied by many downsides. Many expressed a wish for gene therapy.

At the EL-PFDD meeting, patients indicated that they want medications to prevent advanced heart failure, to ultimately avoid or delay treatment/ICD/transplant. Although "gene therapy" was not offered as a response option in the online polls, many patients explicitly expressed their wish for gene therapy to arrest disease progression or to even prevent symptoms from manifesting.

Expert Discussion Point 5

Selection criteria for future gene therapy trials need to be carefully determined.

Experts discussed how gene therapy is a very lengthy and elaborate protocol which may be extremely stressful to endure as it requires multiple tests, many difficult procedures and extended hospitalization.

- The very first patients to be selected for gene therapy clinical trial will likely be higher-risk, symptomatic patients, because gene therapy is a high-risk procedure with uncertain outcomes. These patients will need to have an ICD for both safety and monitoring purposes.

Patients selected for the earliest gene therapy clinical trials will need to have the mental resiliency and adaptability to tolerate the ambiguity of an uncertain result. One expert described these patients as “astronauts”.

- Experts discussed how ideal patient selected for gene therapy (in phase III clinical trials as well as in clinical practice) will need to have enough remaining cardiac function to experience significant benefit if disease progression is arrested. An ideal patient is one who is electrically unstable but has yet to develop cardiomyopathy.
- Although anxiety is not an appropriate clinical trial endpoint, it influences inclusion/ exclusion criteria and informs care during the trial. Patients selected for gene therapy trials need to be able to cope with the procedure, exhibit psychological resiliency and the presence of family/community support, similar to those undergoing a routine transplant workup. Many ARVC patients have extremely high pre-existing levels of anxiety and depression; those with very severe anxiety levels may have to be excluded from gene therapy clinical trials.

Expert Discussion Point 6

Anxiety levels of patients in future gene therapy trials will need to be measured and monitored, and appropriate support provided.

All patients undergoing gene therapy need to have their anxiety measured at baseline and psychological health must be monitored throughout the study. Patients need to be provided with the right type of psychological and medical support for the duration of the study. Experts described how relationship-based psychological support essentially creates a team to walk along side and with the patients throughout their therapy. Employing a standard of care anxiety treatment approach will avoiding confounding.

Expert Discussion Point 7

Although research into ARVC gene therapy is promising, realistic expectations about timelines, treatment opportunities and outcomes are necessary.

Experts are unanimously excited by the potential of gene therapy and the recent gene therapy results demonstrating that restoration of cardiac PKP2 expression in ARVC mice can halt or slow ARVC disease progression[1]. Experts cautioned that many years of hard work are required before gene therapy reaches ARVC patients, and described just a few of the milestones.

- **Current gene therapy models are currently only focusing on the most commonly affected gene in ARVC, plakophilin 2 (*PKP2*).** Gene therapy with desmoplakin gene variants (*DSP* and *DSG2*) is lagging behind, as the genes are too large to fit inside the AAV gene therapy vector. Researchers are trying to determine if using smaller, more essential parts of the gene can work for gene therapy.

[1] Sheikh F, Zhang J, Wang J, et al: Abstract 13599: LX2020, an Adeno Associated Viral-Based Plakophilin 2 Gene Therapy Stabilizes Cardiac Disease Phenotype in a Severe Mouse Model of Arrhythmogenic Right Ventricular Cardiomyopathy. Circulation 146:A13599-A13599, 2022

- **Much more research is still required in animal models**. Researchers need to demonstrate that gene therapy prevents exercise-induced heart damage in animal models and that therapy validated in sedentary mice will continue to hold in animal exercise models.
- **Only after safety in humans is proven can efficacy be established**. After treatment efficacy is demonstrated in mouse models, the safety in humans must be proven. These animal trials will be followed by human trials to demonstrate short and long-term safety and efficacy in humans.
- **Realistic expectations are needed for what this therapy can achieve and the ideal time for therapy delivery determined**. Gene therapy can only arrest disease progression but cannot restore damaged cardiac cells. Researchers must determine how late in the ARVC disease process gene therapy can be delivered and still demonstrate a benefit.
- **Researchers need to determine who is most likely to benefit from the treatment**. Ideally, genetic markers can be identified which can help to stratify patients who are most likely to benefit from the therapy.
- **Appropriate gene therapy endpoints still need to be determined.**

Voice of the ARVC Community

Families want a treatment that will prevent disease from developing in asymptomatic individuals.

ARVC is a family disease. During the EL-PFDD meeting, many from the ARVC community described how genetic testing led to the identification of ARVC variants in children and other asymptomatic individuals. Families worried about future disease progression in younger adults, teens and even children who have inherited an ARVC gene variant, and desire a treatment for gene-positive, presymptomatic patients.

Expert Discussion Point 8

Gene therapy is not yet a consideration for asymptomatic ARVC patients.

Experts agreed that while an important treatment goal is to treat young, unaffected individuals to prevent disease manifestations, the science is not yet at a point where this is possible. In addition to the some of the important work that needs to be completed with gene therapy (described in point 6 above), much additional work is necessary before providing therapy to asymptomatic individuals.

(1) Much more research is required to understand how specific variants may or may not impact the function of the protein in the heart. Researchers need to determine whether each individual ARVC gene variant is pathogenic and will manifest the disease. As an example, the likelihood of developing ARVC in patients who have a *plakophilin 2* (*PKP2*) gene variant, may be as low as 30-50%. Although this is highly dependent on exercise level, there are other factors that need to be understood.

(2) More research needs to be done to explore therapeutic approaches including gene therapy for patients with other gene variants including those for desmoplakin (*DSP*), desmoglein-2 (*DSG2*), plakoglobin (*JUP*), and desmocollin (*DSC2)* and others.

(3) Researchers need to determine if the benefits of treatment will outweigh the risks before treating asymptomatic individuals who have a gene variant. This not only holds for gene therapy, but for other therapies as well.

Acknowledgements

The SADS Foundation would like to thank the many individuals and organizations who helped make both the ARVC EL-PFDD meeting and the Adjunct Scientific Workshop a reality.

First, we extend our heartfelt gratitude to all of the patients and families who participated on June 20th, 2023 EL-PFDD. Thank you for initiating the important conversations and for providing the patient voice to help inform clinical trial design and therapeutic development. Thank you for also joining us for the the July 20, 2023 Adjunct Scientific Workshop.

Thank you to our expert clinician scientists including Dr. Michael Ackerman, Dr. Hugh Calkins, Dr. Mario Delmar, Dr. Samuel Sears, Dr. Wojciech Zareba, and genetic counselor Brittney Murray.

Thank you to our workshop moderators Genevie Echols and James Valentine, as well as to both James Valentine and to Larry Bauer for their guidance throughout the entire EL-PFDD process.

Thank you, Dr. Michael Ackerman, President of the SADS Foundation, for being a part of the Adjunct Scientific Workshop and for providing a summary of the key points.

Thank you to our sponsors who supported the EL-PFDD meeting and the Adjunct Scientific Workshop, including LEXEO Therapeutics, Pfizer, Rejuvenate Bio, Rocket Pharmaceuticals and Tenaya Therapeutics.

Thank you to the many representatives from industry, federal agencies, and physician scientists from across the world who are striving towards a better understanding of the basic and translational science behind this disease and are working towards novel treatments for this disease.

Our hope is that the EL-PFDD Meeting and Adjunct Scientific Workshop together will encourage future research and successful new product development for people living with ARVC who urgently need better treatment options.

Report authors and attributions

This report was prepared on behalf of the Sudden Arrhythmia Death Syndromes (SADS) Foundation by Alice Lara, RN, BSN, CEO & President; Marcia Baker, MS Ed, Program Director; Anna Goodson, Communication Director; and Genevie Echols, RCIS, Family Support Director, all from the SADS Foundation, and by Chrystal Palaty, medical writer.

We respectfully request that the SADS Foundation is acknowledged if any part of this report is used or adapted. For any questions related to this report, please contact Marcia Baker, MS Ed, Program Director (SADS@SADS.org).

Consulting Partners include Larry Bauer, RN, MA, and James Valentine, Esq. and from Hyman, Phelps & McNamara, P.C.

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Workshop Disclosures

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Appendix 1: Additional Resources

FDA's PFDD Guidance Series

https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical

https://www.fda.gov/regulatory-information/search-fda-guidance-documents/demonstrating-substantial-evidence-effectiveness-human-drug-and-biological-products

FDA's Draft Guidance on Substantial Evidence of Effectiveness for Human Drug and Biological Products

https://www.fda.gov/media/133660/download

Appendix 2: Adjunct Scientific Workshop Agenda

July 20, 2023: 12:00 – 2:00 pm Eastern time

1. **Opening Remarks** – Genevie Echols, SADS Foundation

2. **EL-PFDD Summary: What's Important to ARVC Patients & Families** - Larry Bauer, RN, MA

3. **Integrating the Patient Voice into Drug Development** - James Valentine, JD, Workshop Moderator

4. **Opportunities for Patient-Centered Treatment Approaches in ARVC** – Hugh Calkins, MD

5. **Landscape Analysis of Tools to Evaluate What's Important to ARVC Patients** – Sam Sears, PhD

6. **Discussion: Setting a Patient-Focused Research Agenda for Drug Development** (60 min)
   - Hugh Calkins, MD
   - Mario Delmar, MD, PhD
   - Brittany Murray, MS, GCG
   - Sam Sears, PhD
   - Wojciech Zareba, MD, PhD

7. **Key Take-aways** – Michael Ackerman, MD, PhD