

# 2021 RRE

## Agenda (Thursday)

### DAY 1 - Thursday, April 8th

<b>10:00 – 10:15 am</b>	<b>Meeting Welcome &amp; Updates</b>	
	10:00 – 10:05 Welcome	Laura Thrall
	10:05 – 10:15 Meeting Goals, Deliverables	Dr. Caitlin Grzeskowiak

<b>10:15 – 11:25 am</b>	<b>2021 Challenge and Overview</b>	
	10:15 am – Presenting the challenge – <i>Getting a therapy to a patient when they don't fit a specific category.</i> Therapy can be approved regardless of proximal cause (analogy to fever) vs. etiological pathway. Both are possible as pathways for epilepsy. When should you do one versus the other?	Drs. Nathan Fountain & Jacqueline French
	10:35 am – High level overview of seizures vs. etiology vs. syndrome vs. group	Dr. Elaine Wirrell
	10:50 am – Patients & Family Partners Impact Panel	Allison Watson

<b>11:25 – 11:35 am</b>	<b>BREAK</b>	
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<b>11:35 – 12:10 pm</b>	<b>Session I: Syndromes vs. Seizures Therapeutic Approaches</b>	Dr. Jacqueline French
	11:35 am – Anti-Seizure Medication (ASM) vs. Disease Modifying (DMEM) vs. Targeted-Mechanism Epilepsy	
	11:50 am – Discussion	

<b>12:10 – 12:35 pm</b>	<b>FDA Panel</b>	Drs. Billy Dunn & Nick Kozauer
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<b>12:35 – 1:10 pm</b>	<b>Lunch</b>	
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<b>1:10 – 2:40 pm</b>	<b>Session II: Seizure types – Pathology and physiology</b>	
	<i>Following seizure types are select examples due to time constraints, not comprehensive</i>	Dr. Peter Crino Dr. Eleonora Aronica
	1:10 pm – Introduction to the session	
	1:15 pm – Etiology, Pathology & Pathogenesis	
	<ul style="list-style-type: none"> <li>• Infantile spasms &amp; epileptic spasms</li> <li>• Focal seizures, 2 years and above</li> <li>• Generalized and bilateral Tonic-clonic seizures</li> <li>• Tonic and Atonic seizures</li> </ul>	Dr. Jaideep Kapur & Dr. Michael Rogawski

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1:45 pm – Pharmacology/Physiology of each seizure type

- Infantile spasms & epileptic spasms
- Focal seizures, 2 years and above
- Generalized and bilateral Tonic-clonic seizures
- Tonic and Atonic seizures

**Drs. Peter Crino,  
Eleonora Aronica,  
Jaideep Kapur &  
Michael Rogawski**

2:10 pm – Panel Q&A

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**2:40 – 3:30 pm**

**Session III: Considerations for Treating Syndromes**

2:40 pm – Syndromes Overview –  
How homogeneous/generalizable are syndromes? When  
should syndromes respond similarly to therapies? When  
would we expect them to have diverse treatment  
response?

**Dr. Rima Nabbout**

3:00 pm – Specific Syndrome Examples – How are they  
defined.

**Dr. Joseph Sullivan**

- LGS (symptomatology)
- JME (electro-clinically and genetically)
- Dravet (genetically)
- Are therapies specific to one syndrome? Are  
there converging pathways for the therapeutic  
metrics that work?

**3:15 pm – Panel Q & A**

**Drs. Rima Nabbout,  
Joseph Sullivan, Helen  
Cross & Sameer Zuberi**

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**3:45 – 4 pm**

**Session IV: Epidemiology, Clinical Presentation, and  
treatment responsiveness**

*Following seizure types are select examples due to time  
constraints, not comprehensive*

3:45 pm – Infantile spasms & epileptic spasms  
Lecture – Epidemiology and Impact on Pathway /  
Treatment Response

**Dr. Kelly Knupp**

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**4:00 – 4:10 pm**

**BREAK**

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4:10 – 5:20 pm

**Session IV: Epidemiology, Clinical Presentation, and treatment responsiveness**

*Following seizure types are select examples due to time constraints, not comprehensive*

4:10 pm – Focal seizures, 2 years and above

- Lecture – Epidemiology and Impact on Pathway / Treatment Response

**Dr. Daniel Friedman**

4:25 pm – Generalized and focal to bilateral seizures

- Lecture – Epidemiology and Impact on Pathway / Treatment Response

**Dr. Robert Wechsler**

4:50 pm – Panel Q & A

- Discussion – Is this seizure type sufficiently homogeneous to study by itself most of the time? What are circumstances when it wouldn't be studied by itself?

**Drs. Kelly Knupp,  
Daniel Friedman, &  
Robert Wechsler**

# 2021 RRE

## Agenda (Friday)

### DAY 2 – Friday, April 9<sup>th</sup>

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8:30 – 8:40 am	Quick Refresher of Day 1	Drs. Jacqueline French & Nathan Fountain
8:40 – 9:10 am	<b>Session IV: Epidemiology, Clinical Presentation, and treatment responsiveness</b> <i>Following seizure types are select examples due to time constraints, not comprehensive</i>	
	8:40 am – Tonic and Atonic Seizures <ul style="list-style-type: none"><li>Lecture – Epidemiology and Impact on Pathway / Treatment Response</li></ul>	Dr. Sudha Kessler
	8:55 am – Discussion – Is this seizure type sufficiently homogeneous to study by itself most of the time? What are circumstances when it wouldn't be studied by itself?	
9:10 – 9:40 am	Wrap up - How should we study these seizure types	Drs. Kelly Knupp, Daniel Friedman, Robert Wechsler, Sudha Kessler, Dennis Dlugos
9:40 – 11:20 am	<b>Session V: Considerations for Trial Design</b>	
	9:40 am – Basket trial experience outside of epilepsy	Dr. Emil Kakkis
	9:55 am – Statistical considerations for basket trials	Dr. Kert Viele
	10:10 am – How to include a combination of syndromes in a basket trial that would be informative but is not a Phase III? <ul style="list-style-type: none"><li>What are challenges, special considerations?</li></ul>	Dr. Dimitrios Arkilo
	10:25 am – Panel Discussion – using basket trials outside of the syndrome framework. <ul style="list-style-type: none"><li>What grouping should be considered?</li></ul>	Drs. Viele, Nabbout, Sullivan, Arkilo, Rogawski & Kakkis
	10:45 am – Discussion around implications for recruitment for trials <ul style="list-style-type: none"><li>Where can syndromes be combined in trials and where can't they?</li><li>What are ways to group?<ul style="list-style-type: none"><li>pragmatic/phenotypically</li><li>mechanistically</li><li>for impact on seizures only</li></ul></li></ul>	Drs. Gail Farfel, Julia Tsai, Daniel Friedman
	11:00 am – Discussions around implications for analysis & development	Drs. Kert Viele & Nathan Fountain

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**11:20 – 11:30 am**

**Break**

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**11:30 – 12:15 am**

**Wrap up and conclusions of Main Agenda**

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**12:15 – 1:00 pm**

**Lunch**

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**1:00 – 2:30 pm**

**Hot Topics / Late Breaking Topic**

**Drs. Auerbach,  
Perucca, Bergfeldt,  
Welty & Vossler**