February 27 – 28, 2020
French Embassy, Washington, DC

Critical Care Clinical Trialists Workshop

Preliminary program – All speakers are tentative

Faculty

Antoni Bayes-Genis (Barcelona, Spain)
Laura M. Beskow (Nashville, USA)
Sean P. Collins (Nashville, USA)
Deborah J. Cook (Hamilton, Canada)
Martha A. Q. Curley (Philadelphia USA)
Bruno François (Limoges, France)
Etienne Gayat (Paris, France)
Michelle Gong (New York, USA)
Maya Guglin (Lexington, USA)
Michael Harhay (Philadelphia, USA)
Samir Jaber (Intesive Care Medicine, France)
Jacob C. Jentzer (Rochester, USA)
Mikhail N. Kosiborod (Kansas City, USA)
Maciej Kostrubiec (EMA, Poland)
Pierre-François Laterre (Brussels, Belgium)
Pascal Leprince (Paris, France)
Bruno Levy (Nancy, France)
John Marshall (Toronto, Canada)
Michael Matthay (San Francisco, USA)
Alexandre Mebazaa (Paris, France)
Rhonda Monroe (Martinsburg, USA)
William W. O'Neill (Detroit, USA)
Marc S. Penn (Akron, USA)
Peter Pickkers (Nijmegen, The Netherlands)
Susanna Price (London, UK)
Lora Reineck (NHLBI, USA)
Todd Rice (Nashville, USA)
Yves Rosenberg (NHLBI, USA)
Eileen Rubin (Northbrook, USA)
Naoki Sato (Kawasaki, Japan)
Wesley H. Self (Nashville, USA)
Matthew W. Semler (Nashville, USA)
Stuart Spencer (The Lancet, GBR)
Norman Stockbridge (FDA, USA)
Holger Thiele (Leipzig, Germany)
Alison E. Turnbull (Baltimore, USA)
Lorraine Ware (Nashville, USA)
Jayna Williams (Shirley, USA)
Uwe Zeymer (Ludwigshafen, Germany)
Bram Zuckerman (FDA, USA)

THURSDAY, FEBRUARY 27

08:30 am 12:00 pm	**Session 1: Trial design of Cardiogenic shock: experts' recommendations** *Moderators: B Zuckerman (FDA, USA), A Mebazaa, W O'Neill* **Objectives:** *Cardiogenic shock is one of the deadliest diseases in medicine. It is also related to many diseases and the severity is rather diverse. The objective of the session is to agree on a common and global definition and common design of trials***.** **Speakers** ***15 minutes each*** ▪ **Definition, severity** JC Jentzer ▪ **Rescue therapies** M Guglin ▪ **Trial design in the cath lab** U Zeymer ▪ **Trial design in the ICU** S Price **Discussants** ***10 minutes each*** ▪ **Mega-studies = dilution treatment effect** B Davison ▪ **Are observational studies on ECMO relevant?** P Leprince ▪ **Metabolic path: the missing link?** M Kosiborod ▪ **Blocking humoral agents** K Bourgeois (4TEEN4) ▪ **TUSCANI trial** M Penn ▪ **Patient representative** R Monroe ▪ **NHLBI point of view** NHLBI ▪ **Regulators point of view** FDA, EMA
12:00 pm 1:00 pm	**LUNCH BREAK**
1:00 pm 2:30 pm	**Session 2: Global fight to survive from Cardiogenic shock** *Moderators: A Bayes-Genis, S Price* **Objectives:** *Trials in cardiogenic shock are often neutral. Design was often not optimal. The objective of the session is to interact with the investigators of ongoing trials in cardiogenic shock to better design future trials* **Speakers** ***10 minutes each*** ▪ **Ongoing trials in the US** W O'Neill ▪ **Ongoing trials in France** B Levy ▪ **Ongoing trials in Europe** H Thiele ▪ **Ongoing trials in Asia** N Sato ▪ **Post-acute management** G Cotter ▪ **Industry point of view** ▪ **NHLBI point of view** NHLBI ▪ **Regulators point of view** FDA, EMA

2:30 pm 4:30 pm	**Session 3: ED and Critical Care Research: Balancing Human Subjects protection with Meaningful Trial Design** *Moderators: S Collins and W Self* **Objectives:** *Trials in the critically ill need to include an increasing number of patients. However, quality in conducting the trial, especially protection of the subject should remain a key objective. The objective of this session is to cover crucial issues related to trial design and human subjects protection in trials in patients with critical illness.* **Speakers** ***12 minutes each*** ▪ **Alterations in Informed Consent - Answering Important Questions in the Critically Ill** M Gong ▪ **Pragmatic Research and Step Wedge Trial Design** M Semler ▪ **Institutional Review Board Perspective on Minimal Risk Studies and Waiver of Consent** T Rice ▪ **Considerations of Human Subject Protections in Trials in the Critically Ill** – L Beskow **Discussants** ***10 minutes each*** ▪ **Industry** ▪ **Patient representative** Jayna Williams ▪ **NHLBI point of view** ▪ **Regulators point of view** FDA, EMA
4:30 pm 4:50 pm	**COFFEE BREAK**
4:50 pm 6:30 pm	**Session 4: Patients-trialists-regulators cross-talk: what are the meaningful endpoints?** *Moderators: S Jaber (Intensive Care Medicine), S Spencer (Lancet)* **Objectives:** *Prior studies in the intensive care setting have been associated with high mortality and trials were focused on survival. Yet, despite improved survival, patients suffer from very poor quality of life in the weeks and months following an ICU stay. The objective of the session is to join forces among stakeholders to identify meaningful endpoints for future therapies.* **Speakers** ***15 minutes each*** ▪ **Short-term endpoints**: D Cook ▪ **Long-term endpoints** A Turnbull **Discussants** ***10 minutes each*** ▪ **Patient representative:** E Rubin ▪ **FDA:** N Stockbridge ▪ **EMA:** M Kostrubiec ▪ **Post-ICU outcome** E Gayat ▪ **Industry** M Borentain (BMS) ▪ **NHLBI:** Y Rosenberg

FRIDAY, FEBRUARY 28

8:00 am 10:00 am	**Session 5: Interface between ARDS-septic shock is artificial** *Moderators: M Matthay* **Objectives:** *ARDS and septic shock are deadly disease processes often encountered in the ICU. Most trials are focused on assessing benefits of therapies in one or the other disease. However, ARDS and septic shock are highly linked. The objective of the session is to explore common ways to improve outcome in ARDS and sepsis.* **Speakers** *15 minutes each* ▪ **Subphenotypes in sepsis and ARDS** L Ware ▪ **Contemporary therapies and design** M Matthay ▪ **Novel factorial design** M Curley **Discussants** *10 minutes each* ▪ **Industry** ▪ **New trial design** M Harhay ▪ **Patient representative** E Rubin ▪ **NHLBI point of view** L Reineck **Regulators point of view** FDA, EMA
10:00 am 10:30 am	**COFFEE BREAK**
10:30 am 1:15 pm	**Session 6: Novelties in trials in septic shock** *Moderators: PF Laterre, J Marshall* **Objectives:** *Most trials in sepsis were neutral and many promising drugs have been abandoned. However, benefits seen in subgroup analysis suggest that some drugs may have had beneficial effects. Trials design and conduct have suffered from many limitations in the last decade. The objective of the session is to see how to best learn from the past to optimize future trial design.* **Speakers** *15 minutes each* ▪ **Drugs abandoned despite positive results** J Marshall ▪ **RCT in critical care in the past 20 years: what lessons?** S Jaber ▪ **How to best conduct septic shock trials?** PF Laterre ▪ **Are we ready for pragmatic trial in sepsis?** **Discussants** *10 minutes each* ▪ **Adrenoss-2:** A Mebazaa ▪ **ASTONISH: SOFA score as primary endpoint:** B François ▪ **Alkaline phosphatase:** P Pickkers ▪ **Angiotensin:** ▪ **NHLBI point of view:** ▪ **Regulators point of view** – N Stockbridge/EMA
1:15 pm 1:30 pm	**Wrap up and discussion about concepts for meeting manuscript**
1:30 pm	**LUNCH BREAK AND ADJOURN**

Writers of the proceedings of the sessions: TBD