

Rocket Advisory Discussion

Research Directions in Fanconi Anemia Beyond the US/EU 2019 Clinical Trials: Potential for Non-Genotoxic Conditioning (NGC) and Hematopoietic Stem Cell Expansion (HSCE).

February 25, 2019. New York. Version: February 14, 2019

Meeting objectives:

- 1) Recognize available therapeutic entities for NGC and HSCE
- 2) Understand clinical feasibility for these modalities, with emphasis on:
 - agents/platforms available for pre-clinical evaluation in 2019/2020
 - required evaluations to enable clinical evaluation in Fanconi anemia
- 3) Gain consensus on the most promising clinical and pre-clinical research directions.

Agenda:

8:00 – 8:30 am	Breakfast	--
8:30 – 8:45 am	Introduction & Welcome	Hans-Peter & Jonathan
8:45 – 9:00 am	Fancolen-I FA Clinical Study : Topline Results	Juan
9:00 – 9:15 am	Fancolen-II and US FA Clinical Study Design : Brief Overview	Jonathan
9:15 – 9:30 am	Genotoxic and non-genotoxic conditioning in FA murine models	Hans-Peter
9:30 – 9:45 am	Non-genotoxic (CD117-targeted) conditioning: pre-clinical basis and clinical results in PID	Agnieszka
9:45 – 10:00 am	Break	--
10:00 – 10:15 am	HSC expansion clinical studies w/AHRA (MGTA-456) in allogeneic HSCT for inherited metabolic disorders	John
10:15 – 10:30 am	HSC expansion studies w/endothelial cell platforms	Joe, Farid
10:30 – 11:30 am	Discussion : non-genotoxic conditioning	All
11:30 – 12:30 pm	Discussion : HSC expansion	All
12:30 – 1:15 pm	Lunch, summary and conclusions	Hans-Peter & Jonathan
1:15 – 1:30 pm	Departures	--