



## Crigler-Najjar Syndrome – Towards clinical development

Tuesday, December 10, 2013

12.00 – 16.00

Institute of Myology, Batiment Babinski, Salle 1

Pitie-Salpetriere Hospital

Boulevard Vincent Auriol 50/52,

Paris

### Meeting Agenda:

Speaker	Topic	Time (min)
Fulvio Mavilio	Welcome/introduction	10
Federico Mingozzi	AAV vector-mediated liver gene transfer	20
t.b.d.	Crigler-Najjar syndrome, natural history	20
Piter Bosma	Gene transfer in the rat model	20
Andres Muro	A novel mouse model for Crigler-Najjar	20
Matthias Hebben	Manufacturing of AAV vectors	20
Federico Mingozzi	Current developments at Genethon	20

#### Discussion

#### Questions & Answers

#### Future clinical trial design

#### Prescreening of subjects for anti-AAV NAb

#### Definition of "next steps"

### Directions:

The Pitie-Salpetriere Hospital is easily reachable with public transportations. Please see the attached map for details on the location of the Batiment Babinski, the meeting venue. The red arrow indicates the entrance to the building.

#### - From CDG Airport:

RER B to Gare du Nord, change to METRO 5 to GARE D'AUSTERLITZ or SAINT-MARCEL.

#### - From Orly Airport:

Orly bus to DENFERT-ROCHEREAU, change to Metro 6 to CHEVALERET.

**Should any problem occur on the day of the meeting, please contact:**

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## Participants:

<b>Name</b>	<b>Position/Affiliation</b>	<b>Email:</b>
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Beatrice Venturi		n/a