

Pr Odile BOESPFLUG-TANGUY



Recherche fondamentale
Essais précliniques



Essais cliniques

Pour qui ? Pourquoi ? Comment ?



**Université Paris
Sorbonne**



**Institute Of
Muscle-Oriented Translational Innovation**



plateforme d'essais cliniques pédiatriques pour les maladies neuromusculaires.

mission

réaliser des essais cliniques dans le domaine neuromusculaire pédiatrique et notamment dans le domaine des thérapies innovantes.



Directrice Odile BOESPFLUG-TANGUY (0,4ETP)

4 Medecins (3,1 ETP)

Andreea Seferian

Silvana De Lucia

Claudia Ravelli

Ruxandra Cardas

10 ARC

2 chefs de projets

Dominique Duchenne

Ferial Touri

3 Kinés

1 ingénieur qualité

2 IDE recherche clinique

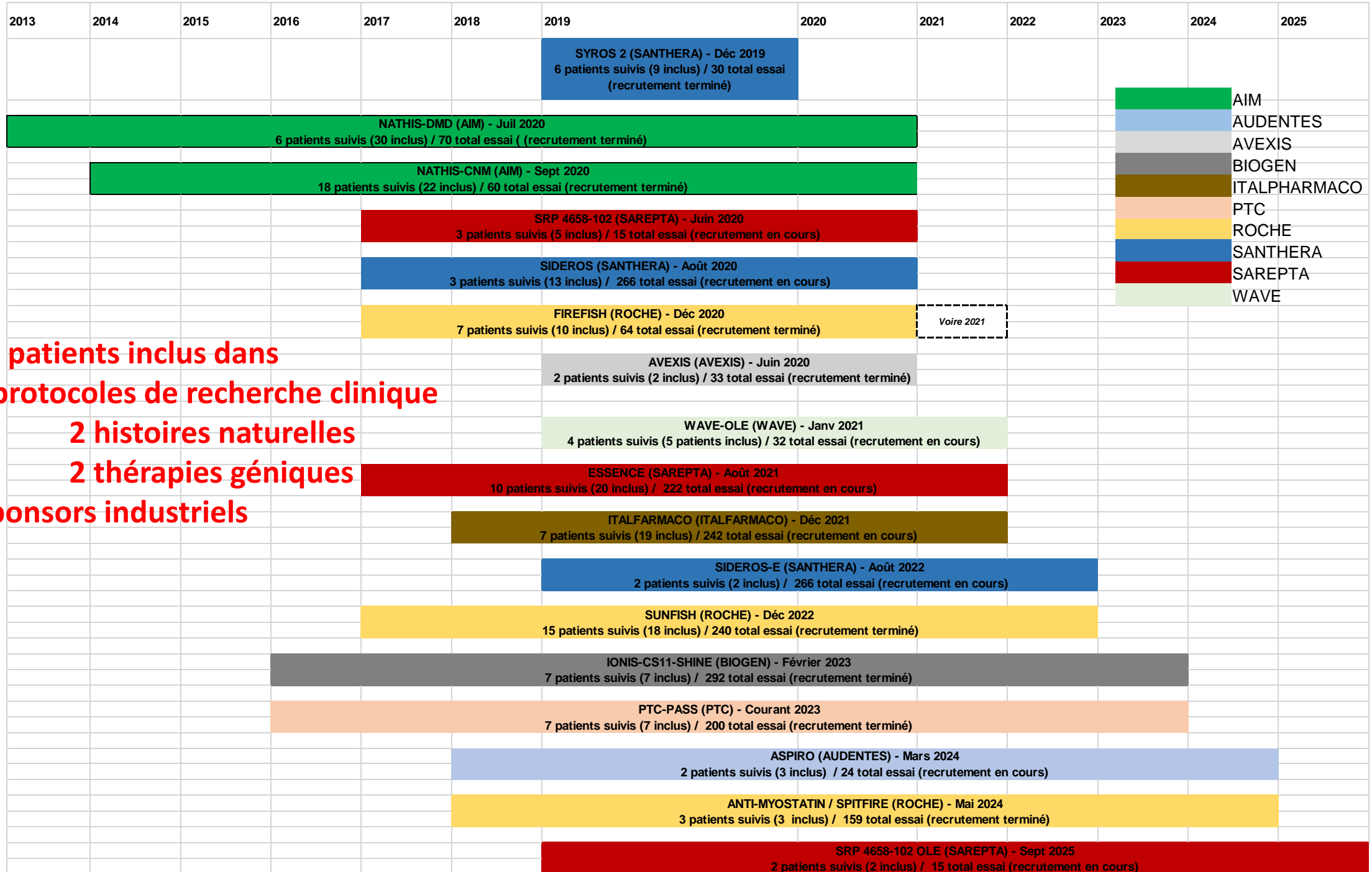
UPARCQ

Psychologue 0,2 ETP

Ergotherapeute 0,2 ETP

1 assistante administrative

1 secrétaire hospitalière



**200 patients inclus dans
17 protocoles de recherche clinique
2 histoires naturelles
2 thérapies géniques
9 sponsors industriels**

- AIM
- AUDENTES
- AVEXIS
- BIOGEN
- ITALFARMACO
- PTC
- ROCHE
- SANTHERA
- SAREPTA
- WAVE



Au sein de Trousseau

Recherche clinique

Pharmacie
Pneumologie
Orthopédie
Ophtalmologie
Cardiologie
Rea



Soins

CRMR Maladies Neuromusculaires
Pneumologie
Rééducation fonctionnelle
Orthopédie
Cardiologie
Rea



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Essais précliniques

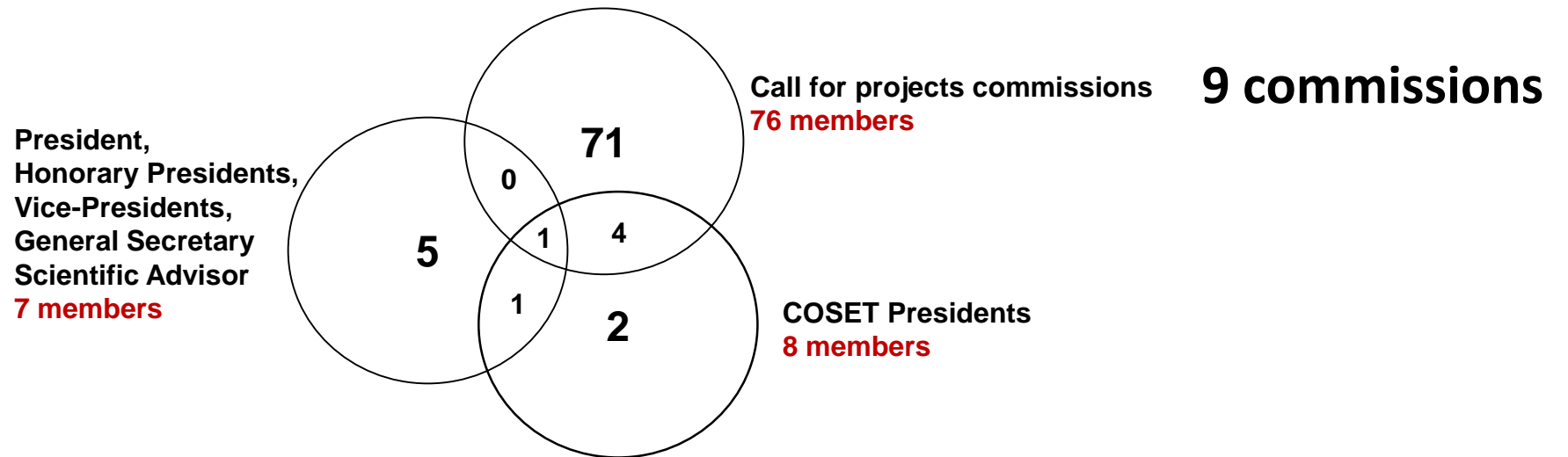


Essais cliniques

The 8th AFM Scientific Council

84 members in 2019

The SC is composed of a President, Honorary Presidents, Vice-Presidents, a General Secretary and a Scientific Advisor, all members of the call for projects commissions, and the presidents of the COSETs (Strategic and Therapeutic Development Committee).



The **Permanent committee** is composed of the President, the Honorary Presidents, the Vice-Presidents, the General Secretary, the Scientific Advisor, the Presidents of the call for projects commissions, and the Presidents of the COSETs (**19 members**).

Its main mission is to address transversal and strategic questions.

Involvement of the Scientific Council in the evaluation and monitoring of the scientific projects and programs

	Strategic Actions	Call for proposals	Other Activities
<p>Bras armés</p> <div style="border: 1px solid black; background-color: #fff9c4; padding: 5px; margin-bottom: 10px;"> <p>Evaluation committee every 2 years including members of the SC and report to the permanent committee</p> </div> <p>YPOSKESI</p> <p>FNA</p>	<p>Strategic Projects</p> <p>Strategic Pole</p> <p>Strategic Tools (4 databases)</p> <div style="border: 1px solid black; background-color: #fff9c4; padding: 5px; margin-bottom: 10px;"> <p>Evaluation a priori by experts including members of the SC and audition of the PI by the permanent committee</p> </div> <p>Each year presentation of the scientific report and COSET or evaluation committee recommendations to the permanent committee</p> <p>Strategic Structures</p>	<p>Institutional Partnerships Program ATIP/Avenir (INSERM/CNRS) INSERM/DGOS</p> <p>Partnerships with patient organizations</p> <div style="border: 1px solid black; background-color: #c8e6c9; padding: 5px; margin-bottom: 10px;"> <p>Evaluation and follow-up by the patient organization SAB</p> </div> <p>Annual call for proposals Research Projects / Fellowships</p> <div style="border: 1px solid black; background-color: #fff9c4; padding: 5px; margin-bottom: 10px;"> <p>Evaluation and follow-up by the Call for projects commissions</p> </div> <p>RNA Call</p> <div style="border: 1px solid black; background-color: #fff9c4; padding: 5px; margin-bottom: 10px;"> <p>Evaluation a priori and follow-up by experts that include members of the SC</p> </div> <p>Other projects</p> <div style="border: 1px solid black; background-color: #fff9c4; padding: 5px;"> <p>Evaluation a priori and follow-up by experts that include members of the SC</p> </div>	<p>Scientific Meetings</p> <p>International networks</p> <p>Animal platform-CEDS</p> <p>Other (Running costs, salaries, expert reimbursement fees...)</p>



Commission médicale
Lien avec les affaires médicales
Réglementaire
Bases de données



Essais cliniques



Au sein de la filiere FILENEMUS

I MOTION structure d'aide à la diffusion et au montage des essais thérapeutiques

Histoires naturelles, biomarqueurs +++

Aide au montage réglementaire

Bases de données

Analyse des données

**Recevabilité pour demande
de financement**

Demandes d'essais

**Sites français doivent être plus lisibles
non en compétitions**

Diffusion du «savoir faire » lors ATU

ARC dédiés I Motion – CRMR / poles strategiques

