



NEWSLETTER 2018

WHAT IS IFCAH ?

International Fund Congenital Adrenal Hyperplasia (IFCAH) is an endowment fund created to fight against Congenital Adrenal Hyperplasia (CAH).

Congenital Adrenal Hyperplasia or CAH is a genetic disease related to a lack of production of adrenal hormones.



Message of the president of IFCAH's scientific committee

Professor Michel Polak (Pediatrician, Endocrinologist, Hôpital des Enfants Malades, Paris, France)

"The IFCAH scientific committee is dedicated **to select the best projects worldwide** that will benefit to the patients and families affected by congenital adrenal hyperplasia."

IFCAH scientific committee

-Pr. M. Polak (FR) -Pr. F. Ahmed (UK) - Dr. A.Martinez (FR)
- Pr. A. Biason-Lauber (CH) - Pr. S. Wudy (GER) - Pr. A. Bachelot (FR)



SOME NUMBERS

2010

Creation of **IFCAH** in **Paris** by parents of children affected by CAH. It aims at giving a strong support to research dedicated to improve the quality of life of patients affected by the disease.

2.2M€

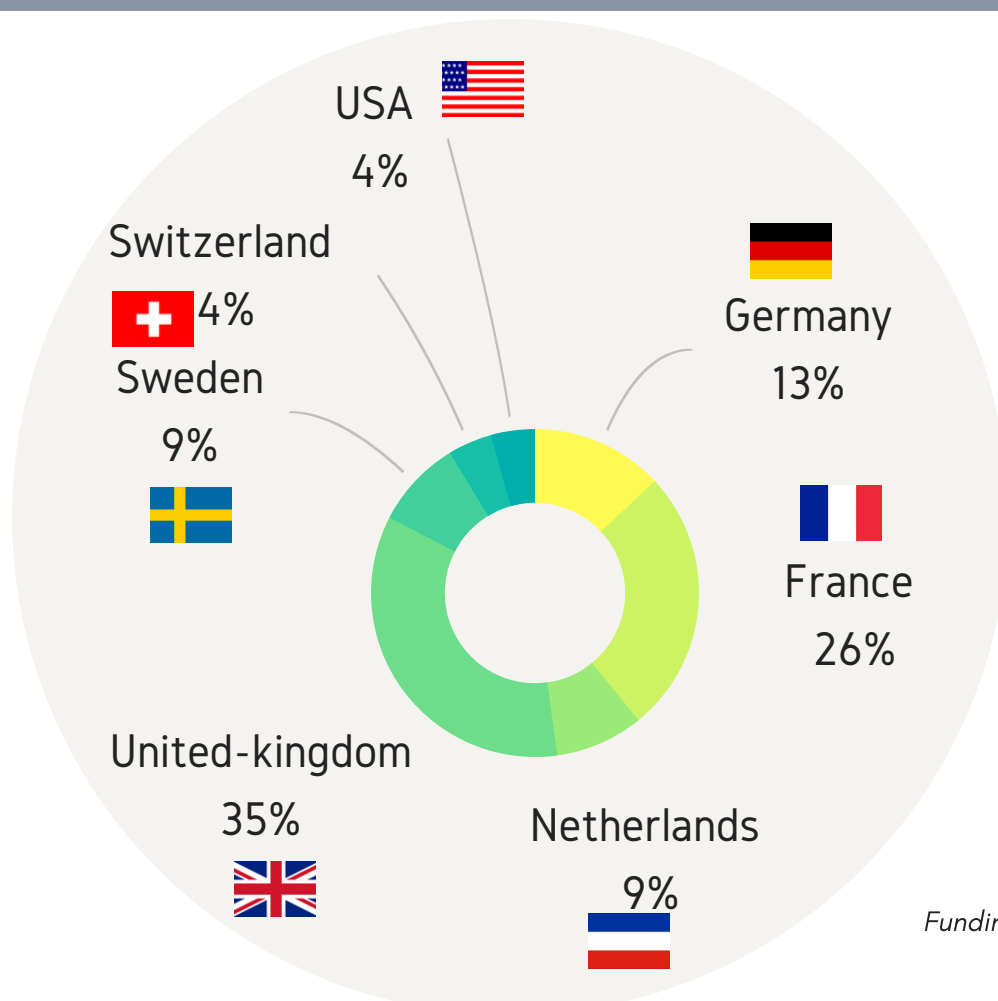
Global amount allocated by IFCAH to the research against CAH since its inception. A total of 26 projets were funded during the 7 calls for proposal from 2010 to 2018.

112

Number of projects submitted to IFCAH during the 7 calls for proposals. Proposals originated from various European countries (France, Germany, United Kingdom, Sweden....) and USA were received.



GEOGRAPHIC DISTRIBUTION OF FUNDED PROJECTS



Funding granted by IFCAH since 2010

IFCAH receive project proposals from more than **19 countries**.



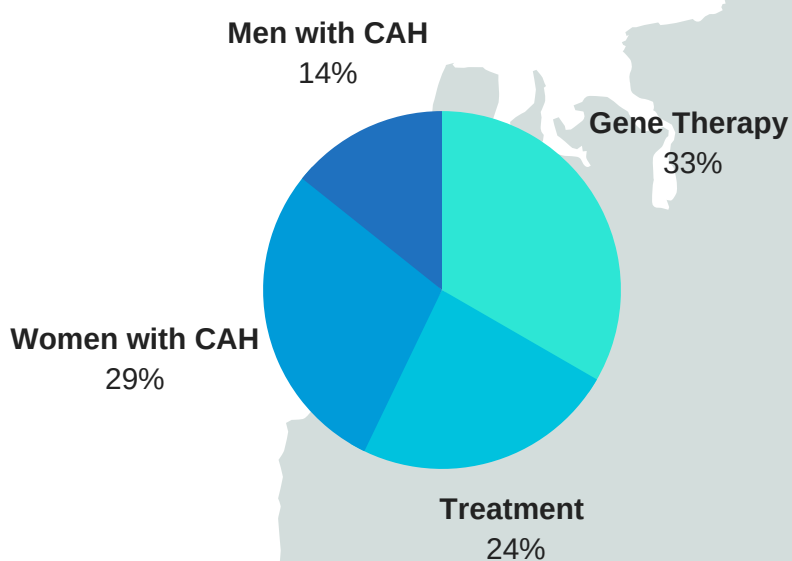
New : IFCAH continues to **expand internationally**, the participation is **open worldwide**; however, if possible, a collaboration with a European team will be appreciated.



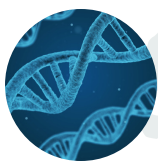
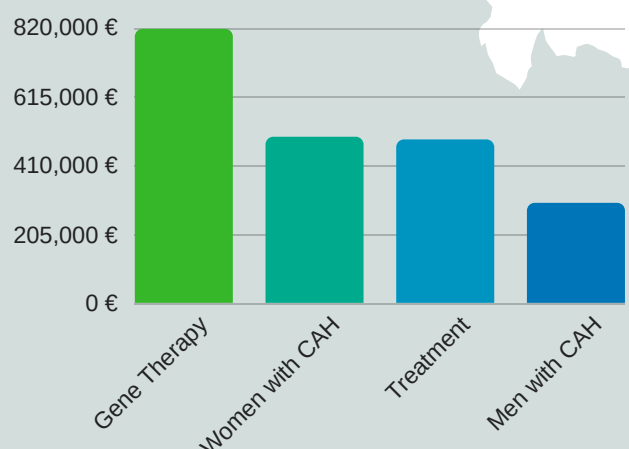
THE SCIENTIFIC RESEARCH

DISTRIBUTION OF FINANCED PROJECTS GROUPED BY RESEARCH TOPIC

Distribution of funded projects since 2010

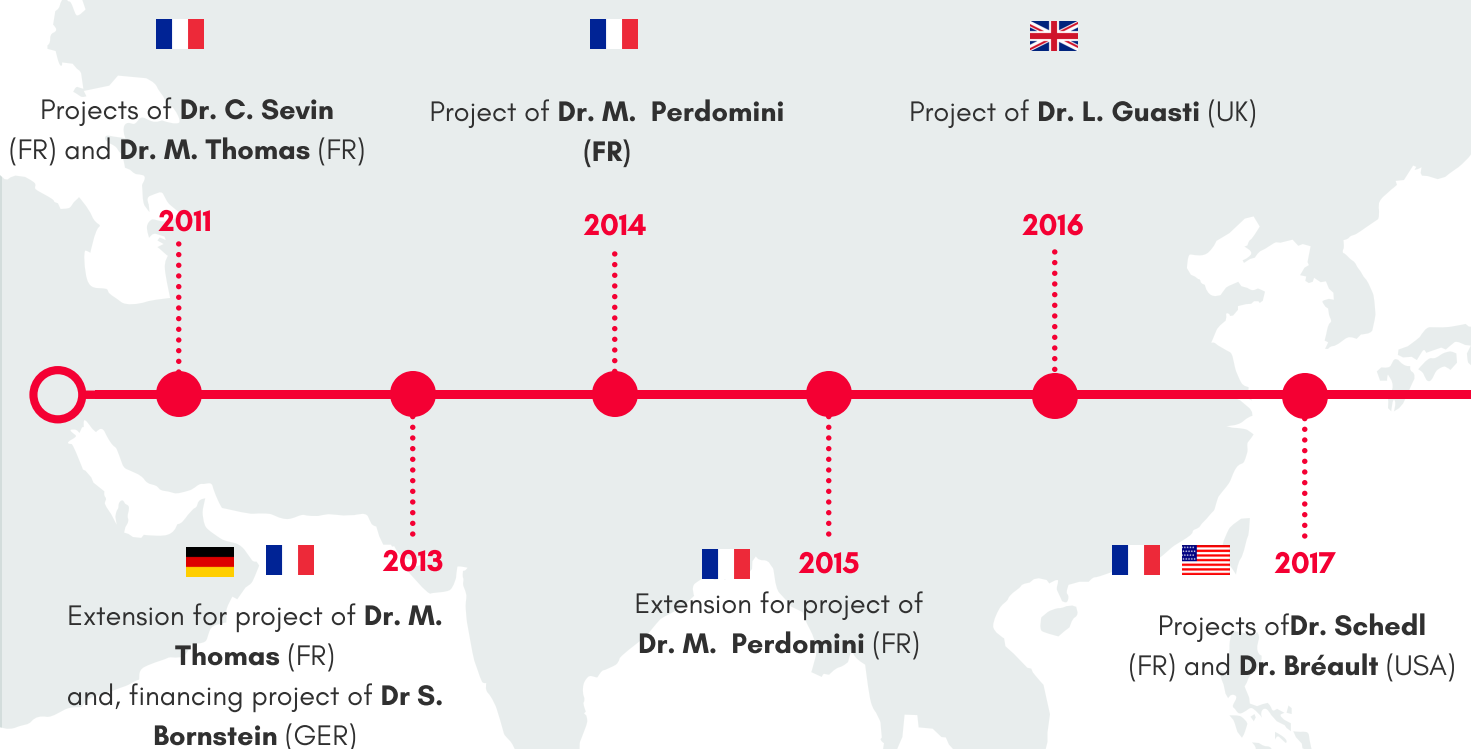


Funding granted by topic since 2010



GENE AND CELLULAR THERAPY

IFCAH and AFM-Telethon have funded **7** gene and cellular therapy projects :



Further details on www.ifcah.org



FOCUS ON RESULT :

" **Gene therapy for the most severe cases of congenital adrenal hyperplasia** " by Dr. M. Perdomini and Dr. P. Bougnères, Le Kremlin Bicêtre, France

Funding from IFCAH allowed an important advance: **almost healing of disease in mouse models of CAH**. Through this project, researchers have proven the therapeutic effects of a gene vector AAV on human gene with adrenal steroidogenesis gene, hormones and clinical parameters (weight, stress resistance) of mouse. The vector of gene therapy, produced in Nantes by Véronique Blouin's team, was administered systemically.

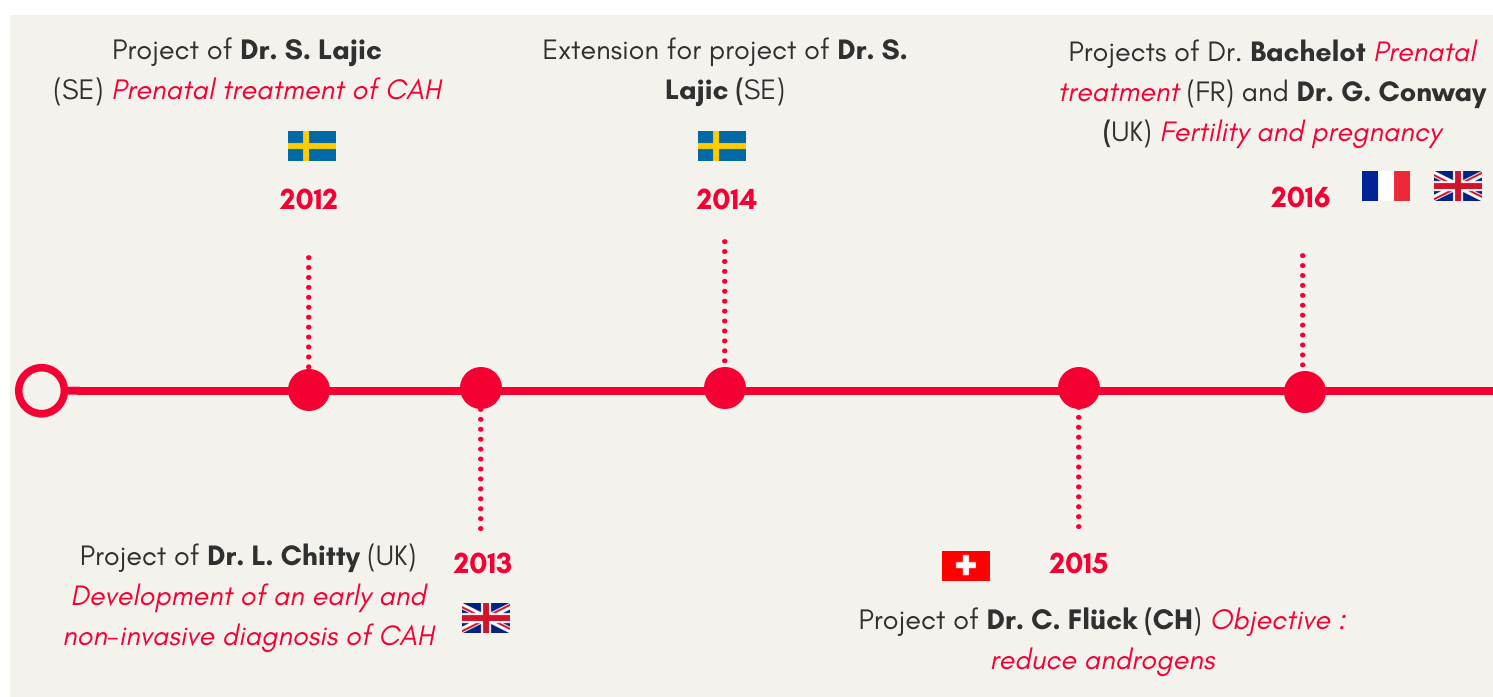
Dr. Bougnères' team is currently studying the best way to lead the program towards patients.

A publication in the journal Gene therapy on February 6, 2016 is related to this project.



WOMEN WITH CAH

IFCAH has funded **5** projects related to women with CAH :



Further details on www.ifcah.org



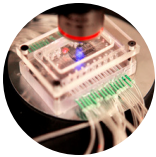
FOCUS ON RESULT :

" **Prenatal treatment of CAH, research for long-term effects on the genome, metabolism and cognitive functions** "

by Dr. Svetlana Lajic, Stockholm, Sweden :

Indeed, **this project has demonstrated the need for prenatal treatment of CAH by dexamethasone in pregnant women.**

Today, the debate is not closed but the trend is rather in favor of this treatment.



MEN WITH CAH

IFCAH has funded **3** projects related to men with CAH :



Project of **Dr. A. Swain** (UK) *Mechanisms involved in the proliferation of intra-testicular adrenal inclusions*

2013

2012



Project of **Dr. H. Claahsen** (NLD) *Male fertility disorders related to the presence of intra-testicular adrenal tissue*

2016



Continued project of **Dr. H. Claahsen** (NLD)

Further details on www.ifcah.org



FOCUS ON RESULT :

" **The etiology and characteristics of adrenal testicular tumor** "

by Dr. H. Claahsen (Nijmegen, Netherland) :

With funding from IFCAH, Dr. H. Claahsen was able to characterize, for the first time, **benign intra-testicular adrenal inclusions or TARTs.**

This work is continued in order to identify the expression patterns of mRNAs and proteins in these TARTs and possible regulation by ACTH. The two main objectives of this project are : **understanding the elements triggering the formation of TARTs and finding targets' treatment for TARTs.**

A publication in the journal JCEM 2014 is related to this project.



IMPROVING TREATMENTS

IFCAH has funded **7** projects with as target to improve current treatments :



Project of **Dr. A. Zimmermann** (GER)

2011



Project of **Dr N. Reisch** (GER)

2013



2016

Extension for the project of **Dr. R. Ross** (UK) and project of **Dr. Martinerie** (FR)

2012

Project of **Dr. N. Krone** (UK)



2015

Projects of **Dr. R. Ross** (UK) and **Dr. R. Stimson** (UK)



2017

Project of **Dr. N. Krone** (UK)



Further details on www.ifcah.org



FOCUS ON RESULT :

" **Cardiovascular risk assessment in patients over 40 years of age with CAH** "

by Dr. N. Reisch, Munnich, Germany:

IFCAH's fund allowed Dr. N. Reisch to have an innovative position. Indeed, unlike previous published studies, his study indicates that **CAH patients have no increase in carotid artery thickness compared to healthy subjects.**

However, some results indicate that **CAH patients may have changes in blood pressure**, especially at night, hence the recommendation for monitoring in CAH patients..

CALL FOR PROJECTS 2019



IFCAH continues to expand internationally...

This year, projects originating from **all around the world are eligible**.

A collaboration with a European team is appreciated.



IFCAH selection criteria...

- Scientific quality of the project
- Feasibility regarding the project's length
- CV of the candidate
- Quality of the technological and scientific environment at the candidate's disposal
- Adequation with IFCAH's objectives

Calendar

October 2018

2018 Call for proposal

January 2019

Deadline to submit the letter of intent

February 2019

Pre-selection among the candidates

April 2019

Deadline to complete the summary of the project

June 2019

Audition by the scientific committee

July 2019

Final selection

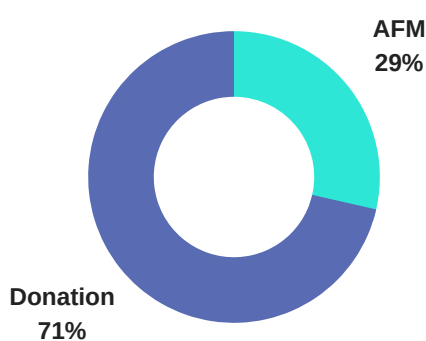


IFCAH needs your generosity !

Funds raised will be exclusively and entirely used to fund research projects on CAH.

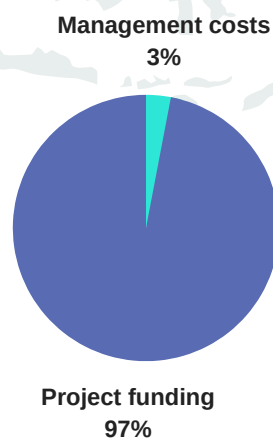
Please visit our website www.ifcah.org for further information.

Sources of funding for 2017



AFM-téléthon IFCAH's partner since 2013, contributes to fund projects that involve genetic and cellular therapy. It contributes to IFCAH projects with an amount of up to **€ 100,000** per year.

Estimate of IFCAH's budget for 2018



Management costs include :

- Legal fees
- Accounting and bookkeeping expenses
- Various financing costs and fees



CONTACT



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PARTNERSHIP