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FSHD Research Funding Overview 2016

This report provides an overview of the worldwide funding of research in the field of Facioscapulohumeral Muscular Dystrophy (FSHD) in 2016 and previous years

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Wouter Suurmond
Corporate development Manager
Facio Therapies BV

Important information

This overview depends on data from different sources using different formats, including public information as well as data directly reported by funding agencies. Therefore - although compiled with greatest care and effort - Facio Therapies BV cannot control or guarantee the completeness and accuracy of this data. The trends shown in this report are based on short project descriptions or titles of grants, therefore these trends are subject to interpretation and might not completely cover all expenses in each specific area of research. This overview will thus at best serve to be an *estimation* of total amounts and trends in FSHD funding. Omissions are deeply regretted.

All amounts are in US dollars. Where necessary, currency conversion were performed using the average exchange rates over 2016:

1,00 EUR = 1,10656 USD
1,00 AUD = 0,74366 USD

Please also bear in mind that the expenses to FSHD research can sometimes be a complex combination of grant commitment and expenditures over several years. Such data collection remains prone to include some errors, missing grants or double calculations. Collecting data in a consistent manner, for example by using the format in the distributed spreadsheet, could help towards improving the accuracy of the overviews.

1. Introduction

This overview serves to estimate the total expenses for FSHD research in 2016. In addition, trends in FSHD research funding are identified where possible. We hope this report serves as a valuable insight in the developments in this important research area. As for the previous reports, the goal is to evaluate the *actual expenses* for FSHD research.

We would like to thank all agencies for their transparency in sharing their FSHD research expenditures, and we would like to especially thank the organizations that used the reporting format that has been distributed.

Content

2. Total FSHD research expenditures

The total estimated research expenditures sum up to over $13,4 million. This is more than previous years, see figure 1. Many funding agencies from all over the world have contributed to this grand total. Their individual contributions are shown in figure 2. The largest funder is the NIH with a contribution of 63% of the total amount of expenses towards FSHD research.

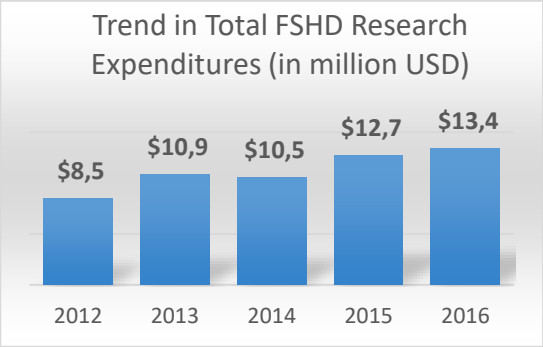

Figure 1: *Total FSHD research expenditures in 2012-2016.*

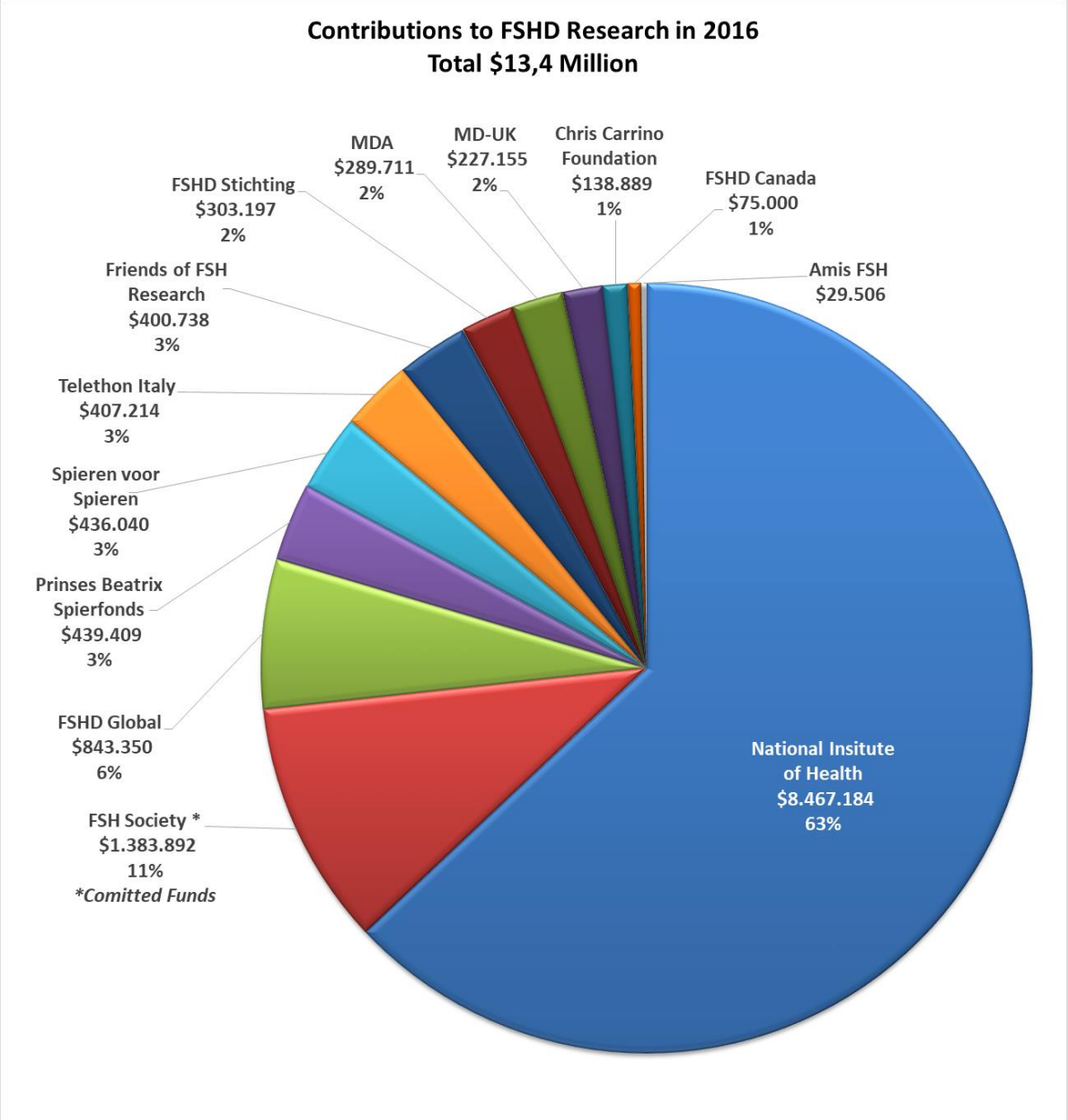

Figure 2: *Contributions of different funding agencies to FSHD research funding in 2016.*

3. Trends in FSHD research funding

3.1 Expenses per stage of research

The expenses per stage of research in 2016 and previous years are shown in figure 3. Most of the expenses are directed towards Fundamental Research & Potential Therapies, this amount is slightly higher than the previous years. A smaller growing part of the expenses is directed towards clinical trial preparedness. Expenses towards animal models for FSHD seem to have slightly decreased in 2016 compared to the years before. Funding of clinical trials makes up a small portion of the total.

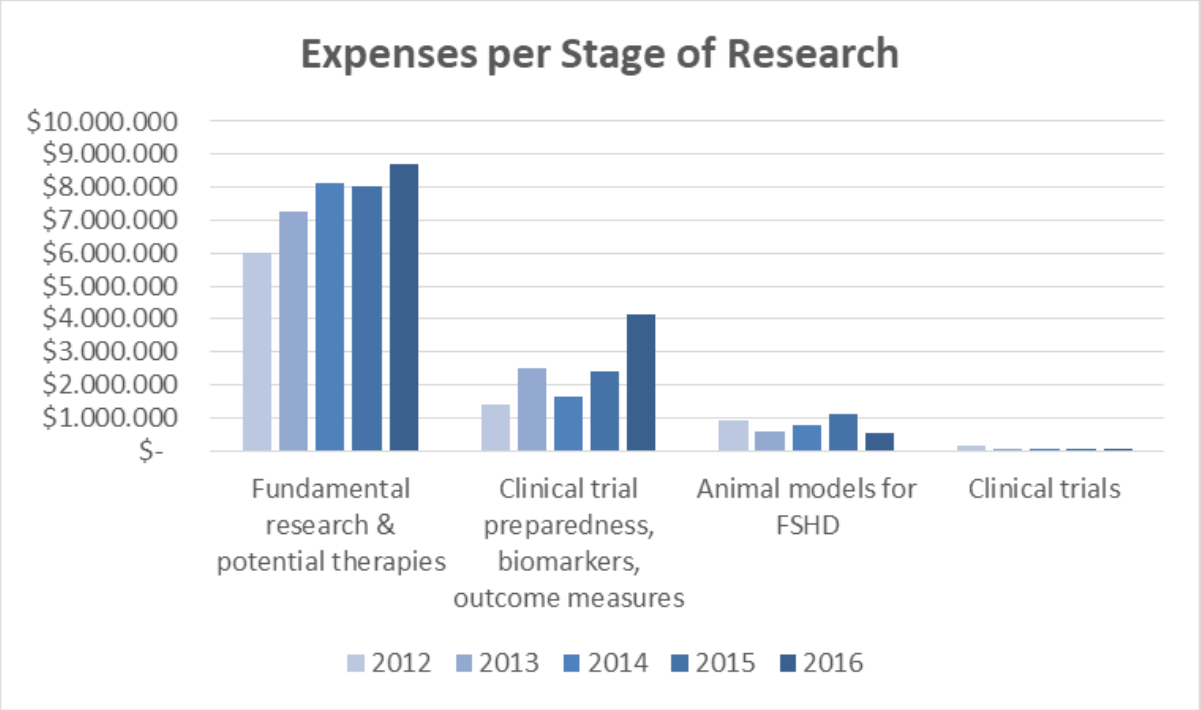

Figure 3: *Trends in the stages of research being funded during 2012-2016.*

3.2 Expenses per therapeutic strategy

The expenses per therapeutic strategy are shown in figure 4. Where possible, distinctions were made between upstream pathways of DUX4, direct targeting of DUX4, downstream pathways of DUX4, , or other targets. Expenses towards upstream pathways of DUX4 are highest and growing compared to the years before. Expenses towards directly targeting DUX4 seem to have slightly decreased. Expenses towards downstream pathways of DUX4 have increased. Small expenses towards research on other targets (FAT1 & FXR1) were identified in 2016.

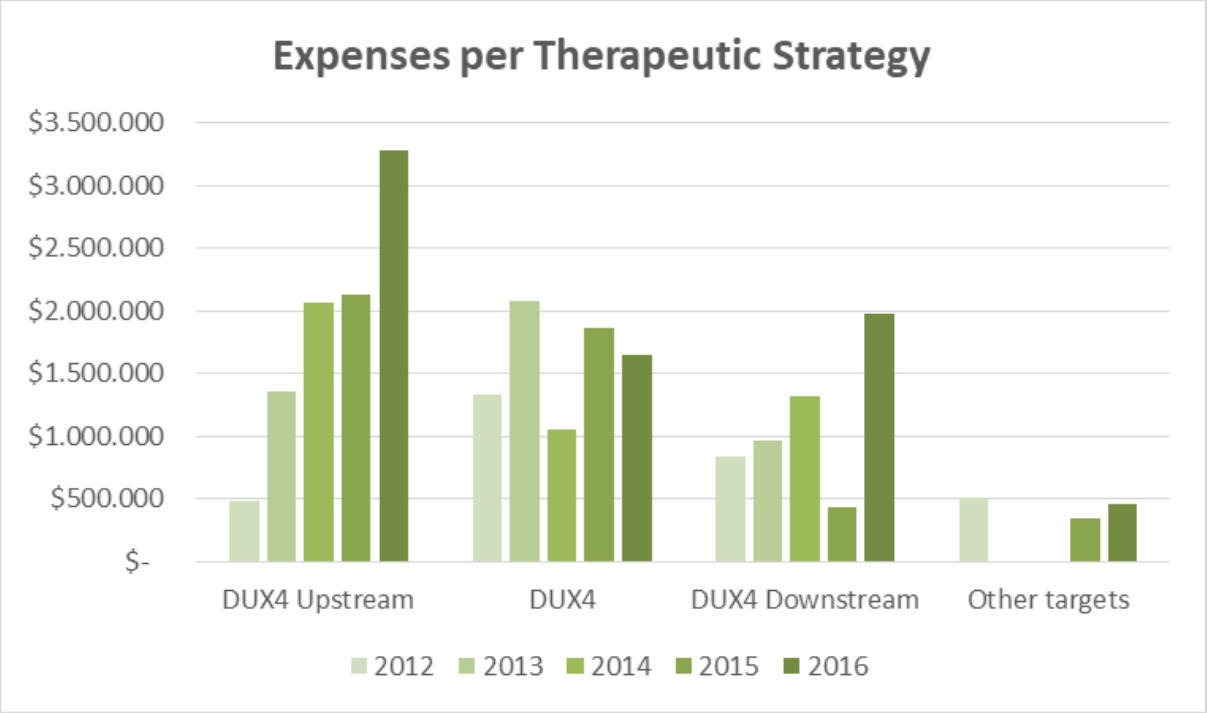

Figure 4: *Trends in the therapeutic strategies being funded during 2012-2016.*

3.3 Expenses per type of therapeutic molecule

Different types of therapeutic molecules exist. Where possible, a distinction was made between expenses towards research on: antisense based therapeutics, biologics, gene editing approaches, and small molecule based approaches. As shown in figure 5, expenses towards small molecule based approaches were highest and have increased compared to the years before. Expenses towards gene editing approached decreased. Expenses towards antisense based therapeutics increased compared to the previous year. Expenses towards biologics are low and less than the previous year.

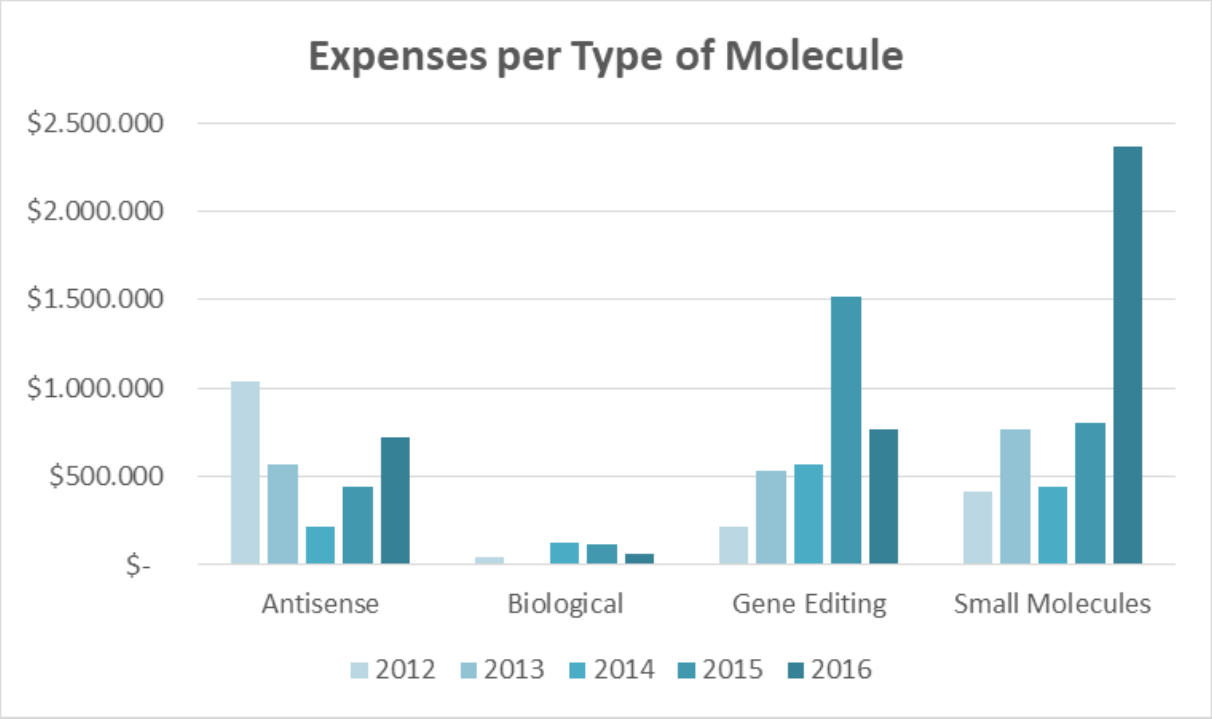

Figure 5: *Trends in the funding of research of different types of therapeutic molecules during 2012- 2016.*