List of projects supported by
the Association Monégasque contre les Myopathies / Only Project
(2013-2023), based on payments.

2023 (*ongoing*)

A. Biotech(s)

In June 2023, SQY Therapeutics launched its phase 1 clinical trial to test the safety of SQY51 – a therapy using antisense oligonucleotides to try to achieve exon-skipping on Duchenne patients concerned with an exon 51 deletion. See more on clinicaltrials.gov
Other projects include the preliminary steps to developing the therapy for other exons (16 and 45) and the acquisition of new material to replace ancient machines.
The biotech is also preparing to move to another facility, outside of the University where it is currently located.

- **SQY Therapeutics**
  - €4 800 000 as treasury advance for budget Q1/Q2 2023.
  - €3 600 000 as treasury advance for budget Q3/Q4 2023.

B. Grants and fellowships

- **Institut Necker**
  - €320 140 for the first increment for a 3-year large scale natural history study (to be renewed in 2024 and 2025) led by Pr. Karim Wahbi on cardiomyopathy associated with Duchenne muscular dystrophy. The charity has already funded the same team to establish a national registry on cardiomyopathies associated with Duchenne muscular dystrophy, which can be expanded internationally over the next three years thanks to renewed funding.

- **Université de Versailles Saint-Quentin-en-Yvelines**
  - €115 000 for a 3-year doctoral grant for student Jeanny Bikouta to study the role of oxytocin in DMD.
  - €100 000 to finance the recruitment for 2 years of an engineer in molecular biology, Lucie Royer, working on the natural history of patients with DMD and Becker muscular dystrophy.

- **Fondation Suisse de Recherche sur les Maladies Musculaires**
  - €204 000 (CHF 196 657) for Mrs Maude Frieden (Université de Genève) and Mrs Gisou van der Goot (EPFL de Lausanne)'s projects through the "Paul Pettavino fellowship" (see more on https://www.fsrmm.ch/fr/projets/projets-de-recherche-2023).

- **Centre Scientifique de Monaco**
    - €8 687 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Support of PhD student Clémence Alibert's** (working on DMD at the Université Claude Bernard Lyon 1 under Pr. Christophe Marcelle's leadership and financed by our association) **participation to the Stem Cell Summer School 2023**:
    - €2 000.

2022

A. Biotech(s)

In 2021 and 2022, SQY Therapeutics achieved preclinical short-term and long-term studies to get drug agencies' authorization for SQY51, the therapy for patients with exon 51 deletion.

- **SQY Therapeutics**
    - €7 000 000 of treasury advance for 2022 budget.

B. Grants and fellowships

- **CNRS / NeuroMyoGene Institute (INMG), Lyon1 University Faculty of Medicine and Pharmacy**
    - €200 000 "Paul Pettavino Fellowship" for PhD student Clémence Alibert's work on "Muscle fusion as a delivery mechanism to repair ailing muscles from heritable muscle diseases" under Pr. Christophe Marcelle's leadership.

- **Fondation Suisse de Recherche sur les Maladies Musculaires**
    - €226 520 (CHF 230 040) for Mrs Marisa Jaconi's work on "Immortal immunoprivileged myogenic stem cells for gene therapy of muscular dystrophy" (2022-2023) through the "Paul Pettavino fellowship".

- **Université de Versailles Saint-Quentin-en-Yvelines**
    - €51 500 for the salary for an Assistant Head of Clinic – Dr. Angely Mendoza Cardozo - from November 01, 2022, to October 31, 2024, as part of our support for the "AVANCE 1" clinical research project led by Professor Amthor at CHU Raymond Poincaré.

- **Centre Scientifique de Monaco**
    - €8 660 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Smart Mobilities & Devices SARL**
    - €36 000 to finance phase 2 of Jean-Paul Carta's 3D simulator to study neuromuscular and neurological diseases in an innovative way.

C. Local patient support

- **Support of Philippes Ferreyrolles, a local patient, for apartment adaptation and mobility expenses**
    - €29 000.

2021

A. Biotech(s)

/

B. Grants and fellowships

- **Université de Versailles Saint-Quentin-en-Yvelines**
    - €100 000 for 24-month salary (2020-2021) of Olga Petkova, engineer, working on the translational research project "tc-DNA therapy for Duchenne muscular dystrophy".
    - €150 000 for Aurélie Goyenvalle's team's work on "Biotherapies for neuromuscular system diseases" and more specifically the "Characterization and development on novel antisense strategies for the treatment of DMD".

- **Fondation Suisse de Recherche sur les Maladies Musculaires / Université de Zurich**
    - €160 800 (CHF 166 800) for Daniela Latorre's project on the "Characterization of autoreactive T-cells in Guillain-Barré Syndrome". Dr. Latorre is a young investigator who was awarded with a PRIMA Fellowship from the Swiss National Science Foundation.

- **Institut Necker**
    - €50 000 for 12-month salary of a clinical research associate within the "Centre de référence des maladies neuromusculaires Nord/Est/Ile de France".

- **Smart Mobilities & Devices**
    - €36 130 to finance phase 1 of Jean-Paul Carta's 3D simulator to study neuromuscular and neurological diseases in an innovative way.

- **Centre Scientifique de Monaco**
    - €9 048 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

C. Local patient support

- **Support of Philippe Ferreyrolles, a local patient, for mobility expenses**:
  - €8 619.

2020

A. Biotech(s)

In 2020, SQY Therapeutics validated its therapeutic principle in vivo. To prepare for preclinical studies and to plan for a clinical trial, the biotech had to supply large quantities of raw materials (tcDNA) and perform production of antisense oligo nucleotides, fill and finish, stability studies, analytics, tags...

- **SQY Therapeutics**
  - €8 711 549 (5 937 173 + 2 774 380)

In 2020, Synthena's chemical activities were transferred to SQY Therapeutics:

- **Synthena**
  - - €2 774 380 (amount reimbursed and reinjected in SQY Therapeutics the same year)

B. Grants and fellowships

- **Novosibirsk State University**
  - €125 000 for a scientific exchange and cooperation agreement with biotech SQY Therapeutics.

- **Université de Versailles Saint-Quentin-en-Yvelines**
  - €57 000 for 12-month salary of Amalia Stantzou, postdoctoral fellow working in the laboratory U1179 on the translational research project « Skeletal muscle pathophysiology – Differential expression and restoration of dystrophin at critical expression sites in the muscle fiber ».

- **École Polytechnique Fédérale de Lausanne (EPFL)**
  - €23 120 (CHF 25 000) for Mohamed Bouri's "REHAssist" work within EPFL and amongst others the development of mobility aids and rehabilitation devices.

- **Centre Scientifique de Monaco**
  - €9 622 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

C. Local patient support

- **Contribution to Philippe Ferreyrolles', a local patient, mobility expenses**
  - €7 000

2019

A. Biotech(s)

Detoxification of the "candidate therapy" and start of preliminary studies for future clinical trial.

- **Synthena:** € 4 233 507 (CHF 4 600 000)
- **SQY Therapeutics :** €2 500 000

B. Grants and fellowships

- **University of Massachusetts Medical School**
  - €155 874 awarded to Pr. Robert Kotin for part of the expenses required for Identifying and characterizing genomic safe harbors in humans and murine genomes for insertion of dystrophin expression cassettes.

- **Institut Necker**
  - €90 000 for Pr. Karim Wahbi's "DMD Heart Study".

- **Université de Versailles Saint-Quentin-en-Yvelines**
  - €70 000 for Aurélie Goyenvalle's team's work on "Biotherapies for the neuromuscular system diseases" and more specifically the "Characterization and development on novel antisense strategies for the treatment of DMD".

- **Biologica Naturalia – Professeur Duboc**
  - €52 500 for Pr. Denis Duboc's work on the use of beta-blockers to preserve DMD patients' cardiac muscle.

- **Charité Medical Faculty of Berlin and the Max Delbrück Center for Molecular Medicine**
  - €10 796 for a short-term postdoctoral fellowship to Dr. Jakub Malcher as part of the French-German research project on the topic of the "Gene transfer efficiency of AAV vectors depending on dysferlin expression".

- **Centre Scientifique de Monaco**
  - €4 961 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

C. Local patient support

- **Contribution to Philippe Ferreyrolles', a local patient, mobility expenses**
  - €7 000

2018

A. Biotech(s)

2016/2017/2018: Search for the right antisense oligonucleotide candidate targeting exon 51 skipping in collaboration between Synthena (chemists) and SQY therapeutics (biologists). Improving the composition of antisense sequences.

  - **Synthena:** €2 024 042 (CHF 2 280 000)
  - **SQY Therapeutics:** €100 000

B. Grants and fellowships

- **CNRS / NeuroMyoGene Institute (INMG), Lyon1 University Faculty of Medicine and Pharmacy**
  - €150 000 for Pr. Christophe Marcelle's work on "Muscle fusion as a delivery mechanism to repair ailing muscles from heritable muscle diseases"

- **CNRS**
  - €50 000 for Dr. Cyrille Vaillend's project titled "Gene therapy to correct cognitive deficits in Duchenne muscular dystrophy".

- **Charité Medical Faculty of Berlin**
  - €30 000 for Prof. Dr. Markus Schuelke, MD et Dr. Mina Petkova, PhD's work (50% of a technical assistant for one year).
  - €11 750 for a short-term postdoctoral fellowship to Dr. Jakub Malcher as part of the French-German research project on the topic of the "Gene transfer efficiency of AAV vectors depending on dysferlin expression".

- **Centre Scientifique de Monaco**
  - €27 346 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Dr. Luis Garcia**
  - €15 000 for a mission to consolidate the SQY Therapeutics – Synthena – Université de Versailles Saint-Quentin-en-Yvelines interface.

- **Institut Imagine for genetic diseases**
  - €1 000 donation

2017

A. Biotech(s)

2016/2017/2018: Search for the right antisense oligonucleotide candidate targeting exon 51 skipping in collaboration between Synthena (chemists) and SQY therapeutics (biologists). Improving the composition of antisense sequences.

- **Synthena**: €1 750 591 (CHF 1 350 000 ; €601 332,37)
- **SQY Therapeutics:** €150 000

B. Grants and fellowships

- **Universitäts-Kinderspital beider Basel (UKBB)**
  - €150 000 to finance the costs of one additional trial site to conduct the multicentric clinical trial titled: “TAMDMD” (Tamoxifen in Duchenne muscular dystrophy). This clinical trial was submitted to the European Union E-Rare joint transnational call for proposals 2016 for "Clinical research for new therapeutic uses of already existing molecules (repurposing) in rare diseases" and had been recommended for funding.

- **Australian Regenerative Medicine Institute (ARMI), Monash University**
  - €100 000 for Pr. Christophe Marcelle’s work on “Muscle fusion as a delivery mechanism to repair ailing muscles from heritable muscle diseases”.

- **Université de Versailles Saint-Quentin-en-Yvelines**
  - €53 000 for Pr. Helge Amthor’s laboratory’s work on “The use of DmdEGFP-mdx reporter mice for studying dystrophin restoration in revertant fibers and following treatment with tricyclo-DNA oligomers-mediated exon skipping".

- **Dr. Karim Wahbi**
  - €35 000 for a one-year grant destined to finance a detachment in London, within the Neurology Service of the Queen Square Hospital (UCL).

- **Centre Scientifique de Monaco**
  - €33 313 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Dr. Luis Garcia**
  - €33 000 for management of the LIA-BAHN Laboratory (UVSQ-CSM Monaco) and scientific consultancy for the association.

- **Charité Medical Faculty of Berlin and the Max Delbrück Center for Molecular Medicine**
    - €23 500 for an intermediate fellowship for four months to Jakub Malcher as part of the French-German research project on the topic of the "Molecular mechanism of the action of tricycle-DNA in dystrophic muscle".

- **Naturalia et Biologia**
    - €7 500 for Pr. Karim Wahbi's "DMD Heart Study".

C. Local patient support

- **Contribution to Philippe Ferreyrolles', a local patient, mobility expenses**
    - €6 493

2016

A. Biotech(s)

2016/2017/2018: Search for the right antisense oligonucleotide candidate targeting exon 51 skipping in collaboration between Synthena (chemists) and SQY therapeutics (biologists). Improving the composition of antisense sequences.

- **Synthena: €**2 000 212
- **SQY Therapeutics: €**200 000

B. Grants and fellowships

- **University of Massachusetts Medical School**
    - €260 248 ($288 441) for Dr. Robert Kotin work to establish re-administratable, non-viral gene transfer using Closed-Ended (ce)DNA that overcomes the major obstacles facing viral vectors.

- **Université de Versailles Saint-Quentin-en-Yvelines / Centre Scientifique de Monaco**
    - €55 000 for the hiring of an engineer over a year, based at the CSM Monaco to assist the LIA-BAHN (Laboratoire International Associé – Biologie Appliquée aux Handicaps Neuromusculaires) team's work.

- **Naturalia & Biologia**
    - €47 000 for the cost of a nine-month fixed-term contract for a clinical research associate collecting clinical and genetic data for the « DMD-Heart-Study » on cardiac involvement in Duchenne muscular dystrophy.

- **Centre Scientifique de Monaco**
    - €19 524 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at

the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Dr. Luis Garcia**
  - €12 000 for a scientific consultancy and mission involving biotech Synthena.

2015

A. Biotech(s)

Synthena's chemistry is mature enough to launch a second biotech, SQY Therapeutics, based in France, that will work on the biology aspects and on bringing the idea to the clinical trial stage.

- **Synthena:** €997 588
- **SQY Therapeutics:** €610 000

B. Grants and fellowships

- **Naturalia & Biologia**
  - €62 400 for the cost of a clinical research associate, on fixed-term contract, for one year, gathering clinical and genetic data for the "DMD Heart Study" on cardiac involvement in Duchenne muscular dystrophy.

- **Centre Scientifique de Monaco**
  - €18 496 for supplies for the Monégasque-French team of the LIA-BAHN laboratory on Biotherapies applied to neuromuscular disabilities, located at the Monaco Scientific Center (CSM) and whose creation was significantly supported by the charity, in 2013.

- **Dr. Luis Garcia**
  - €10 832 for scientific consultancy for the association.

- **Duchenne Parent Project France**
  - €10 000 donation.

C. Local patient support

- **Participation to material needs for local patients**
  €17 000

2014

A. Biotech(s)

2013 and 2014: improvement of manufacturing processes and search for suppliers to increase production capacity.

- **Synthena**
    - €1 509 553

B. Grants and fellowships

- **University of Bern**
    - €150 000 for their contribution to the scientific work on tcDNA.

- **University of Massachussets Medical School**
    - €92 561 ($125 000) for Pr. Robert Kotin (NIH - Washington DC) and Pr. Lee Sweeney (UPENN - Philadelphia)'s project to evaluate the therapeutic benefit of whole-body AAV-U7 gene transfer in GRMD myopathic dogs. The main aim being to determine whether dystrophin rehabilitation is still effective in dystrophic dogs with long-standing disease.

- **King's College London, Randall Division for Cell and Molecular Biophysics**
    - €80 000 for Pr. Simon Hughes' key studies on the evaluation of the therapeutic values of quasi-dystrophins in zebrafish.

- **Dr. Luis Garcia**
    - €32 204 for consultancy for the association (scientific coordination).

- **University of Oxford**
    - €15 000 grant to Pr. Dame Kay Davies (Oxford University - Oxford)

2013

A. Biotechs

2013 saw confirmation of the strong therapeutic potential of antisense oligonucleotides in the tricyclo-DNA (tc-DNA) family developed by Prof. Christian Leumann's team (University of Bern) and for which Synthena is responsible for industrial development. It also saw the improvement of manufacturing processes and the search for suppliers to increase production capacity.

- **Synthena**
    - €2 163 042

B. Grants and fellowships

- **Luis Garcia**
    - €27 080 for scientific consultancy for the association.

- **University of Oxford**
    - €15 000 for Pr. Dame Kay Davies (Oxford University - Oxford) for the provision of relevant mouse models in the context of various preclinical evaluations of

gene therapy and especially "splice switching" in collaboration with the UVSQ and the Synthena company.

C. Local patient support

- **Participation to material needs for patients:**
    - €4 000