21 July 2023

POLICY PLAN
Stichting Friedreich's Ataxia Research Alliance Europe
("**Foundation**")

1. Introduction

The Foundation was established on May 15th 2023 and its registered office is in Amsterdam. The Foundation is registered with the Commercial Register of the Dutch Chamber of Commerce under number *90213602*.

The Foundation's mission is to treat and cure Friedreich's Ataxia (FA) by marshaling and focusing European and global resources and relationships.

FA is a rare inherited condition that affects children and adults. It is caused by mutations in the *FXN* gene which results in deficiency of an essential cellular protein called frataxin. Without enough frataxin, cells in the body have trouble functioning correctly, particularly nerve and heart cells. The primary symptoms include:

- loss of coordinated movement of the upper and lower limbs (ataxia) that leads to loss of ambulation and difficulty performing activities of daily living,
- hypertrophic cardiomyopathy which can lead to arrhythmia and/or heart failure,
- vision and hearing loss, dysarthric (slurred) speech,
- fatigue, scoliosis (curvature of the spine), and diabetes.

It is estimated that there are 7,000-10,000 individuals with FA throughout Europe and 15,000-20,000 globally.

Advances in research and development of treatments for FA could have impact and direct translation to other rare conditions, specifically mitochondrial diseases, hereditary ataxias, cardiomyopathies and neurodegenerative conditions.

The Foundation works collaboratively with the Friedreich's Ataxia Research Alliance (a non-profit corporation incorporated and registered in the District of Columbia United States of America – "**FARA**").

In order to be considered an ANBI (*algemeen nut beogende instelling; public beneficial organization*) by the Dutch tax authorities under applicable tax laws, the Foundation must have an up-to-date policy plan.

This policy plan provides insight in the way the Foundation carries out its work in order to achieve its objects for the next 5 years, elaborating on:

a. the Foundation's objects and the activities that are carried out;
b. the way the Foundation raises funds;
c. management of the Foundation's assets/funds; and
d. the use of the Foundation's assets/funds.

2. The objects of the Foundation

Mission

The Foundation's mission is to treat and cure FA by marshaling and focusing European and global resources and relationships, by advancing treatments that will slow, stop and reverse FA, by empowering researchers and patients and by facilitating clinical research across the globe. Achieving this mission will have a profound impact on the quality of life of those individuals diagnosed with FA as well as family members and caregivers. This mission will be accomplished by raising funds for research, promoting awareness and supporting scientists, patients, clinical networks, government agencies, pharmaceutical companies and other FA organizations that share the same mission. The Foundation shares the common goal with FARA's mission to marshal and focus the resources needed to treat and cure FA.

Short term ambition

One of the first and primary objectives of the Foundation will be to provide operational and financial support to the European sites of the Friedreich Ataxia Global Clinical Consortium ("FA GCC"). The FA GCC brings together clinical research investigators, institutions, and advocacy organizations who have been conducting natural history studies and clinical trials in FA. The goals of FA GCC are to capture a complete view of the natural history of FA in a large patient cohort, generate high quality datasets, establish international standards for clinical outcome assessments, promote collaboration and information sharing across clinical sites and share knowledge and experience with biopharma companies and regulatory bodies. In achieving these goals, the Foundation will be providing the necessary global clinical research infrastructure that facilitates clinical trials and brings treatments to individuals with FA.

Another important initiative of the Foundation is to align with patient advocacy organizations (FA and other rare diseases) throughout Europe to advocate for policies that facilitate and expedite drug development, include the patient voice and experience into each step of clinical development and regulatory decision-making process, and promote access to approved treatments.

Long term ambition

The objectives of the Foundation are to create and support scientific and research programs, like the FA GCC, along with education and awareness activities which

accelerate the development of treatments and cures for FA. Clinical, scientific, and biomedical research programs will be supported through various mechanisms such as funding research grants, organizing seminars and conferences, and growing the FA scientific community.

The Foundation can serve as a patient advocacy hub and convener providing information, educational resources, and communication to other regional patient advocacy groups.

The Foundation aims to facilitate a coordinated and collaborative interaction between all stakeholders: researchers, physicians, patient families, industry and government partners – that leads to an improved FA diagnosis and fast development of treatments for FA. This collaboration is meant to be transnational and will engage international researchers with the global FA patient community.

Activities of the Foundation to achieve its goals

To achieve its objectives, the Foundation cooperates with and supports organizations that are operating for similar purposes. Specifically, the Foundation will collaborate with FARA in establishing the FA GCC, leveraging FARA's resources, experience and knowledge in establishing such large collaborative research initiatives. The Foundation will collaborate with other European advocacy organizations empower patients, families, caregivers, health care providers, the medical community, researchers and scientists, affiliated organizations, and businesses to be advocates for action, and to facilitate and promote research.

The Foundation's strategic priorities and activities are:

- supporting and unifying the clinical infrastructure in Europe and globally to create an FA GCC that informs and accelerates new treatments for FA;
- leveraging existing resources in addition to financial support for the FA GCC creation and sustainability;
- sharing knowledge and know-how, to accelerate the pace of FA research;
- collaborating with all stakeholders: researchers, physicians, patient families, industry and government partners, to achieve the mission to slow, stop and reverse FA;
- developing resources that advance all therapies in the field of FA at the global level.

3. Raising funds

The Foundation raises funds by applying for state subsidies, but also by means of private gifts and donations, including by inheritance or legacy. The Foundation will seek donations and sponsorships from private individuals as well as corporate partners. The Foundation will also apply to public and private agencies or foundations for financial support.

4. Management and use of the Foundation's assets/funds

Management of assets/funds

The Foundation keeps records of all gifts, donations, and assets.

Moreover, the management board will prepare the financial statements within six months after the end of the financial year (which runs from 1 January to 31 December). These financial statements shall also be published on the Foundation's website.

Use of assets/funds

The funds received will be used solely for the implementation of the Foundation's objectives.

Managing directors of the Foundation will not receive any stated salaries. They might be entitled to reimbursement of the expenses incurred by them in the performance of their duties as managing directors with due observance of the ANBI requirements.

The majority of the raised funds will support FA research at the FA GCC sites in Europe and any additional funds will support initiatives aligned with the Foundation's objectives.

Records of the Foundation will be kept according to recognized accounting standards.

5. Organization and governance of the Foundation

The Foundation has a management board that will consist of an odd number of managing directors of at least five (5), the exact number of managing directors to be determined by a resolution of the management board. Thus, there is no individual or legal entity that has majority control over the assets of the Foundation.

The management board may establish committees, in particular (but limited to) project-bound steering committees. Such committees, not being a corporate body, shall primarily act in an advisory capacity regarding all matters concerning the

foundation and the foundation's objects. Only with due observance of the ANBI requirements, a committee member may be entitled to reimbursement of the expenses incurred by them in the performance of their duties.

At the present time no such committee is established.