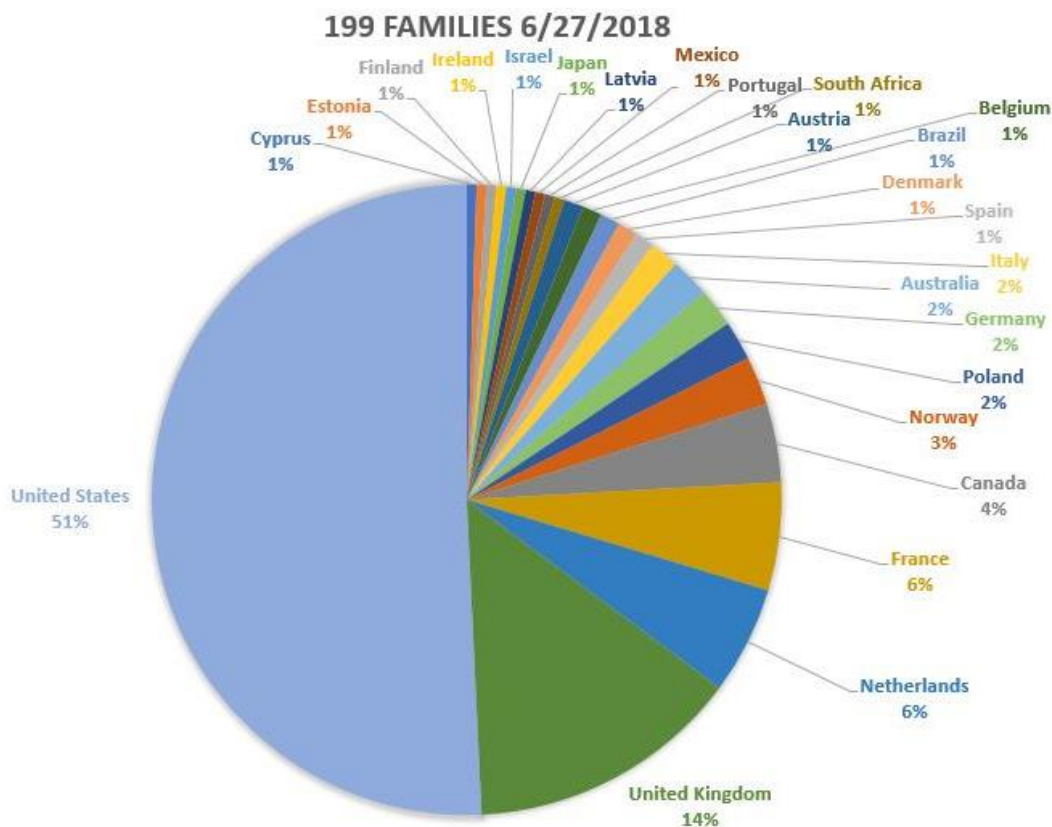


# Executive Summary

In 2008 two patients with an affected DYRK1A gene were identified in a published article to have similar characteristics and a few more articles came after. However, it wasn't until 2012 when it was first reported to be syndromic in patients. In 2015 a spike in published articles identified several individuals with DYRK1A Syndrome. While a few parents waited for the articles to be published they started a Facebook group in August of 2014. Since the beginning of the Facebook group the numbers of individuals with DYRK1A Syndrome has nearly doubled in size every year. By the end of 2017 we reached 156 families across the world that have an individual with DYRK1A Syndrome.



Since DYRK1A Syndrome is an ultra-rare condition and only recently identified, there wasn't an existing organized effort to make an impact on lives of individuals with DYRK1A Syndrome.

**DYRK1A Syndrome International Association (DSIA) will exist to support and maintain a unified community of families and professionals focused on improving the lives of individuals affected by DYRK1A Syndrome.**

With a rapidly growing and globally dispersed group, unity will be the key piece to the growth and knowledge of DYRK1A Syndrome. Though organizations, charities & groups may be

created across the world, being connected to collaborate on common goals will be the most effective way to move forward as a community in advancing what is known about DYRK1A Syndrome.

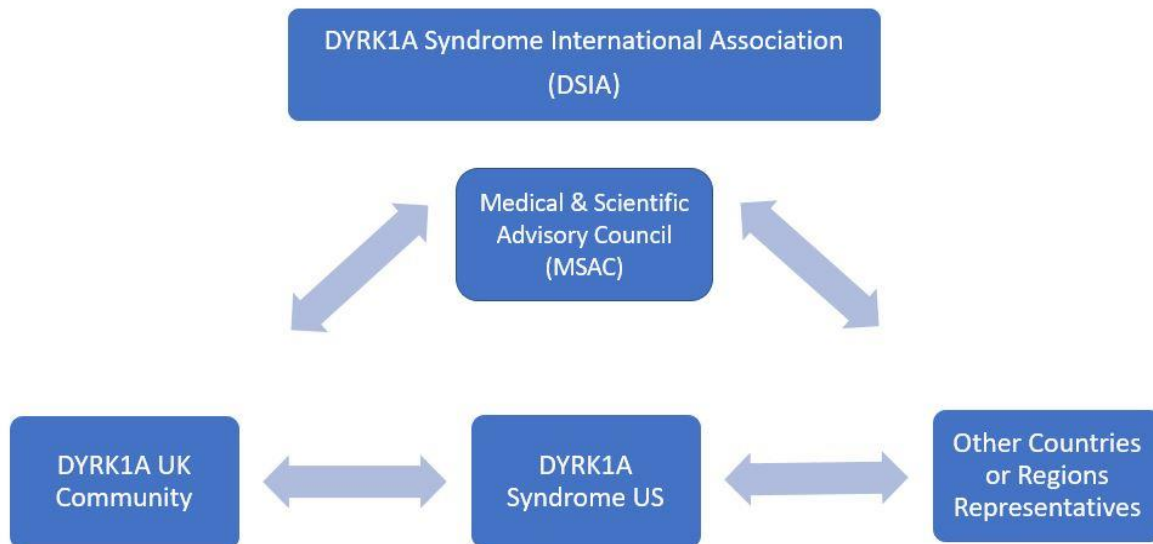
Supporting families is going to be another key factor in the role of the DSIA. It can be very therapeutic for families when they know they aren't alone in experiencing the challenges that may come with having a child with DYRK1A Syndrome. Having the proper support will enable families to better cope with the multitude of challenges of attending to their children's needs, having a direct impact on the entire family as well as the progress of the child. Becoming more knowledgeable about DYRK1A Syndrome can also help families not only in the healing process but also with becoming an advocate for their child's needs. In order to support families on a more local level we will help and encourage creation of new Entities and we will appoint Representatives where Entities do not exist. When families feel adequately supported they are also much more willing to participate in research.

The DSIA will play a role in advancing research and understanding of DYRK1A Syndrome. We currently are in close connections with the Tiger Study, SimonVIPConnect, and GenIDA. Increasing participation in these current research initiatives will improve the overall knowledge of DYRK1A Syndrome. At some point in the future of DSIA, it may be advantageous to look at having our own robust data registry. As we grow and funding becomes more available we will go beyond just encouraging research and participation to being able to fund specific research. We will create a Medical & Scientific Advisory Council (MSAC) to help advise us in this area of our goals. We will plan scientific meetings in conjunction with a Family Meetup rotating locations across the world.

To make our presence known in the world we will brand our identity with a logo that can be utilized by the DSIA, Entities, Representatives and the MSAC. Our website will also be modified to include the existence of Entities and Representatives. We will extend access to our social media outlets to Entity leaders and Representatives to increase our presence to the world. We will encourage families to share their stories or create blogs to share. Handing out informational materials will also bring awareness to DYRK1A Syndrome.

Collaborating with other rare disease organizations and having DYRK1A Syndrome represented in the larger rare disease groups will help bring awareness to DYRK1A Syndrome. It will also help us further our mission and goals by making connections to people who can positively influence our growth.

# Organizational Structure



## DSIA

DYRK1A Syndrome International Association (DSIA) will be governed by a Board of Directors. Board members shall consist of a diverse group of people representing families, professionals, communities or individuals from all over the world. Members shall also be people representing local, regional or country entities. The DSIA Board of Directors shall have the following officer positions; President, Vice-President, Secretary & Treasurer.

## Committees

Committees will be created to focus on specific goals and objectives that have been decided upon by the DSIA Board of Directors. Committees shall consist of at least 1 DSIA Board of Directors and other members of our community. Committees that are likely to be needed; Finance Committee, Program Committee, Awareness Committee & Research Committee.

## Entities

An entity shall be any organization or charity that is created for the sole purpose of improving the lives of more than one individual affected by DYRK1A Syndrome. In order for these entities to utilize shared resources through the DSIA they must commit to supporting the common programs, goals and objectives that they may share with the DSIA. Entities may place a member of their governance on the board of directors of the DSIA.

Entities shall have their own governance and be responsible for any rules and regulations that they are bound by where they are located. They will have their own funding stream and be responsible for their own operating costs. They shall be financially responsible for any programs, goals or objectives that their governance chooses to focus on. They must be willing to consider supporting any of the common programs, goals or services that they may utilize through DSIA. Entities must be willing to consider supporting areas and people that are unserved by an entity.

Currently we have two proposed entities at the beginning stages of becoming formalized. One in the UK and the other in the USA. Other entities will be created in local, regional or countries as needed by the DYRK1A community. Entity creation will be based on whether there are people interested in creating an entity and whether that entity is interested in being involved in shared programs and goals. The DSIA will then discuss and determine whether an entity shall be part of the DSIA.

## Representatives

The area's of our community that are unrepresented by any Entity shall have representation through a representative. Representatives shall be added based on individual's availability and interest in being a representative. The DSIA will also monitor our community and recruit representatives in areas that may present a need for representation. Representatives will stay connected with the DSIA to keep us inform of the needs of DYRK1A families in their country or region. The DSIA will provide information and resources to Representative to help them in their role.

## Medical & Scientific Advisory Council

The Medical & Scientific Advisory Council (MSAC) provides strategic input, insight and expertise to DSIA in the key areas of research, drug development and potential partnerships related to DYRK1A Syndrome. The MSAC mission, goals and advisory role is strategically aligned with the DSIA mission.

This multidisciplinary council serves in an advisory capacity to the DSIA leadership team to further specific initiatives as well as work together to broaden the impact of DSIA by advancing innovation in technology, science, advocacy and research.

The MSAC may consists of MDs, PhDs, MBAs, clinicians, geneticists, scientific researchers and professors.

# Programs, Goals & Services

## International Unity

To maintain unity we will create shared resources that all Entities may utilize. These shared resources may include; Facebook group, Facebook page, website, mailing list, email list, data, We shall expect DSIA, Entities and Representatives to work together in collaboration to create programs and accomplish goals.

## Family Support

We will continue providing international support and resources in the DYRK1A Family & Friends Facebook Group. We may look at other resources to support families who do not utilize Facebook Groups. We will encourage, support and advise on the planning of DYRK1A Family Meetups. Entities will be created and Representatives will be appointed to support families on a more local level.

## Research

We will continue to encourage participation in current research initiatives that focus on DYRK1A. We will create a Medical & Scientific Advisory Council to help advise the DSIA. We will hold a Scientific Meeting annually or biennially.

## Awareness / Marketing

A logo will be created to be utilized by the DSIA, Entities, Representatives and the MSAC. We will improve our website to help with awareness and marketing. We will increase our use of social media to make ourselves more present in the world; Facebook Page, Twitter, Blogs & Mail Chimp Email list. We will create and distribute educational and awareness materials about DYRK1A Syndrome. We will increase the number of locations where DSIA is listed as resource for DYRK1A Syndrome.

## Collaboration

We will look for opportunities to collaborate with other organizations that may help further our mission and goals. We will strive to have some representation with the rare disease organizations across the world. We will build relationships with rare disease leaders and industry leaders to help accomplish this goal.

# Financial Structure

One Entity will be the main holder of the funds directed to the DSIA. These funds will be kept separate from the Entities own budget and finances.

Each Entity may hold the funds that it receives from individuals outside its service area which may be considered an international donation. Each Entity will be expected to budget for any of the common programs, goals and services that it may be utilizing. The Entity's leaders will be part of the planning process for determining the financial needs and priority of certain programs, goals and services.

