We envision a world where every person with Down syndrome thrives with improved health, independence, and opportunities to reach their fullest potential.
About LuMind IDSC Foundation
A history of success that began with involved families

LuMind established in 2004 by a group of families investing in groundbreaking research for Down syndrome, and grew to include other leading foundations (IDSC and RDS).

2004

The LuMind IDSC research approach is focused on collaborative, translational DS research and has supported 15 clinical trials on 1300 participants to date.

1,300

Lumind IDSC has the largest Down syndrome community with 275,000 followers on Facebook, 17 Facebook support groups and an annual in-person event for 4000 family members.

275k

Granted $20M to top academic institutions and biopharmaceutical companies, leading to >$50M in concurrent funding from the NIH, industry and other sources.

70

The research that LuMind has funded has led to the discovery of 10 potential therapeutic targets and the development of 3 assessment scales for children and adults.

13
Our Research Strategy is very “translationally” focused

Prevent AD
- LIFE-DSR natural history
- Cognitive / Functional Assessments
- Industry engagement

Independence
- Cognition
- Sleep
- Behavior
- Speech
- Regression

Gene Therapy
- Gene therapy/editing
- Chromosome silencing

Access to Treatments
- Accelerate Research (DS-CTN)
- Improve Medical Care
Translational Key Opinion Leader (KOL) Meetings
To define with a diverse set of experts the next frontiers to advance promising therapeutic approaches

Our philosophy is to Invest each research $ with a “translational” end-goal in mind and build needed resources/infrastructure.

We accelerate research with a network of world-leading scientists, clinicians and drug developers

JUN 2017
Alzheimer’s assessment scales in Ds

FEB 2018
Gene and chromosome silencing in Ds
Research momentum to address the unmet needs of people with Down syndrome
Top 5 Medical Concerns are Consistent across several recent Parent Surveys

- Nicole White Survey (N=256, Spring 2018)
  1. Cognition/Independence
  2. Speech/communication
  3. Sleep apnea
  4. Alzheimer’s
  5. Behavior/social skills
  6. Hypothyroidism

- LuMind pre-merger survey (N=126, February 2019)
  1. Cognition Development
  2. Independence
  3. Speech
  4. Alzheimer’s
  5. Behavior

- Facebook Research & Medical Care Group (N=210+, Feb-Sep 2019)
  1. Cognition Development
  2. Alzheimer’s
  3. Speech
  4. Sleep Concerns
  5. Behavior

- IDSC pre-merger survey (N=82, February 2019)
  1. Cognition Development
  2. Speech Development
  3. Behavior
  4. Alzheimer’s
  5. Sleep Concerns
2019 BARCELONA
429 Attendees from 36 Countries

2019 BARCELONA
70 Speakers
4 Plenary Lectures
3 Satellite Symposium

2019 BARCELONA
167 Research Posters
65 short talks

2017 CHICAGO
232 Attendees

2017 CHICAGO
60 Speakers
13 Sessions

2017 CHICAGO
71 Research Posters
2 Poster Sessions

2015 PARIS
230 Attendees from 20 Countries

is a Founding Member and Proud Sponsor
Growing DS research momentum with increased NIH funding & multiple cohorts

- NIH Include Project
  2018: $25M+ in additional funding to DS research

- NIH DS Connect Registry
  4,525 members

- NIH ABC-DS Biomarker cohort
  >450 cohort in US

- DS-CTN network
  11 sites in US

- LonDowns cohort
  452 cohort in EU

- DABNI cohort
  485 cohort in EU

- LIFE-DSR cohort
  270 cohort in US

- Human Trisome Project cohort
  520 cohort in US

- Horizon21 network
  7 sites in EU

- NIH ACTC-DS network
  Emerging network

- DS Cognition Project cohort
  ~300 cohort
DS-CTN and LIFE-DSR study in adults
LuMind IDSC 2020 Vision for Alzheimer’s clinical trials in Down syndrome

Lead a collaborative effort to enable rapid enrollment of up to 500 participants for Alzheimer’s clinical trials in Down syndrome by:

1. Establishing necessary infrastructure
2. Developing assessment scales
3. Engaging with pharma companies
4. Rallying the Down syndrome community
LuMind launched in Nov 2018 the Down Syndrome Clinical Trials Network (DS-CTN)

$3.0M in initial commitment

11 sites accepted grant awards to be part of DS-CTN, with >5,000 patients seen annually
DS-CTN Mission:
Improve the lives of individuals with DS

1. Advance effective treatments for people with DS

2. Attract more public and industry sponsors to conduct clinical trials for DS

3. Significantly and tangibly improve the care of those with DS
11 Clinics, Coordinating Center and Central Bio-Bank involved in DS-CTN at launch

website: www.ds-ctn.org
• **Observational**, multi-center, **longitudinal cohort study** to characterize adults with DS ages 25 years and above enrolled at specialized care centers.
  
  o Assess changes in cognition, behavior, function and health
  
  o Develop sensitive and well-validated assessment instruments of cognition, behavior and function appropriate for future clinical trials (**includes two new: Strydom amd Goal Attainment scales**)

• **Study period**: 36 months (first patient first visit to last patient last visit)

• **Enrollment period**: approximately 12 months

• **Participant data collection period**: 24 months (from the 3 clinic visits)

• **Number of participants**: 270 persons

• **Longitudinal data collected**: demographics, clinical, cognitive, behavioral and functional data, and blood collection
The LIFE-DSR Study has received central IRB approval
First site active in August, 3 sites in September, now 7/12 sites activated
45 participants baselined (13) or scheduled (32)
SAS scale for AD in DS: Project Structure

Data Analysis:
Jason Hassenstab & Andy Aschenbrenner (Wash USL)
Judith Jaeger, Consultant

6 EU Sites:
U Cambridge, Institut Lejeune, Hosp. Santa Creu i San Pau, Erasmus MC, DZNE
## GAS Method and Data Value – Clinician and Pharma Perspectives

### GAS PROMOTES ADHERENCE
- Promotes shared decision-making
- Changes the nature of the dialogue between patient and clinician
- GAS is motivating
- GAS can promote adherence

### GAS IS CLINICALLY MEANINGFUL
- GoalNav™ can be used from Phase 1b onward
- More sensitive than traditional outcome measures
- Inherently clinically meaningful and relevant
- Ideal for clinical trials and to generate data for payers

### GAS PROVIDES REAL WORLD EVIDENCE
- By design, GAS incorporates wishes and concerns of patients / caregivers
- GoalNav™ allows for facilitated GAS by trained rater / interviewer
- SymptomGuide® can be used to collect RWE directly from patients / caregivers
New Assessment Scales Timelines and Validation in LIFE-DSR

SAS
Final scale development and LIFE-DSR baseline visit start

GAS
KOL meeting, Establish Menu Items

GAS
LIFE-DSR Validation Sub-Study (Baseline visit)

GAS
Validation Analysis, Study conclusions

SAS
LIFE-DSR Validation Sub-Study (FU visit #1)

SAS
Validation Analysis, Study conclusions

Publication
Publish on both assessments

Note: GAS timeline is pending additional funding for validation part.
Tau PET imaging Collaboration in LIFE-DSR

- Collaboration announced with Cerveau Technologies in May 2019
- Intention to perform tau PET scans on 50 of the LIFE-DSR participants at 2 time points (100 total scans) to help define Alzheimer’s progression.
- Exploring additional funding through industry and/or NIH grant.
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