



LuMind IDSC Foundation: Supporting Research for Alzheimer's in Down syndrome

SweDown Conference in Stockholm

October 3, 2019

Hampus Hillerstrom, President and Chief Executive Officer

We envision a world where every person with Down syndrome thrives with improved health, independence, and opportunities to reach their fullest potential.



1

About LuMind IDSC Foundation



A history of success that began with involved families

2004

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).

1,300

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

275_k

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

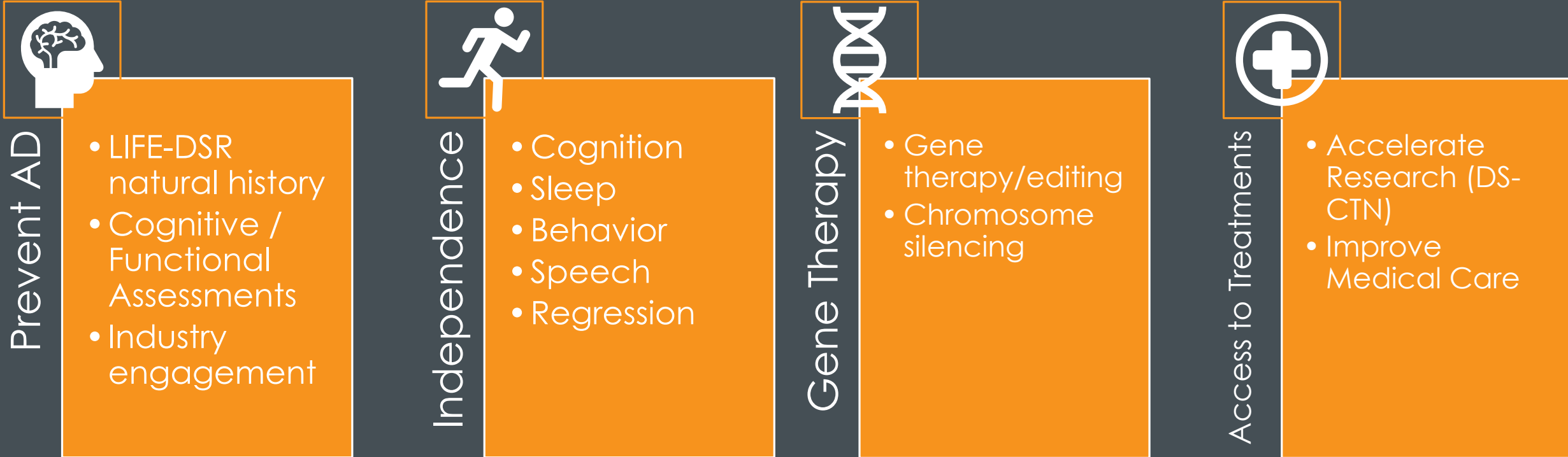
70

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

13

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

Our Research Strategy is very “translationally” focused



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



The image shows a group of professionals in a meeting room. A presentation screen on the left displays a slide titled "LUMIND Research Down Syndrome Foundation: Translational Key Opinion Leader Meeting: 2018 Symposium on Down Syndrome February 6, 2018". The slide features a photo of a child and the LUMIND logo. The text on the slide reads: "LUMIND Research Down Syndrome Foundation: Translational Key Opinion Leader Meeting: 2018 Symposium on Down Syndrome February 6, 2018". Below this, it says "LUMIND: The successful foundation of research into therapies to support people with Down syndrome and health for individuals with Down syndrome".

Translational Key Opinion Leader (KOL) Meetings To define with a diverse set of experts the next frontiers to advance promising therapeutic approaches	JUN 2017 <i>Alzheimer's assessment scales in Ds</i>	FEB 2018 <i>Gene and chromosome silencing in Ds</i>
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Our philosophy is to Invest each research \$ with a “translational” end-goal in mind and build needed resources/infrastructure.

2

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

○ Facebook Research & Medical Care Group (N=210+, Feb-Sep 2019)

1. Cognition Development
2. **Alzheimer's**
3. Speech
4. Sleep Concerns
5. Behavior

○ LuMind pre-merger survey (N=126, February 2019)

1. Cognition Development
2. Independence
3. Speech
4. **Alzheimer's**
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

2019 BARCELONA

included
Industry Session
Education Cmte Session
Science & Society Session

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

DS-CTN network
11 sites in US

LIFE-DSR cohort
270 cohort in US

NIH DS Connect Registry
4,525 members

LonDowns cohort
452 cohort in EU

Human Trisome Project cohort
520 cohort in US

NIH ABC-DS Biomarker cohort
>450 cohort in US

DABNI cohort
485 cohort in EU

Horizon21 network
7 sites in EU

NIH ACTC-DS network
Emerging network

DS Cognition Project cohort
~300 cohort

3

**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

Coordinating Center



Bio-Bank



Central IRB



Longitudinal Investigation for Enhancing Down Syndrome Research (LIFE-DSR) Study

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

LIFE-DSR – Status?

- 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



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

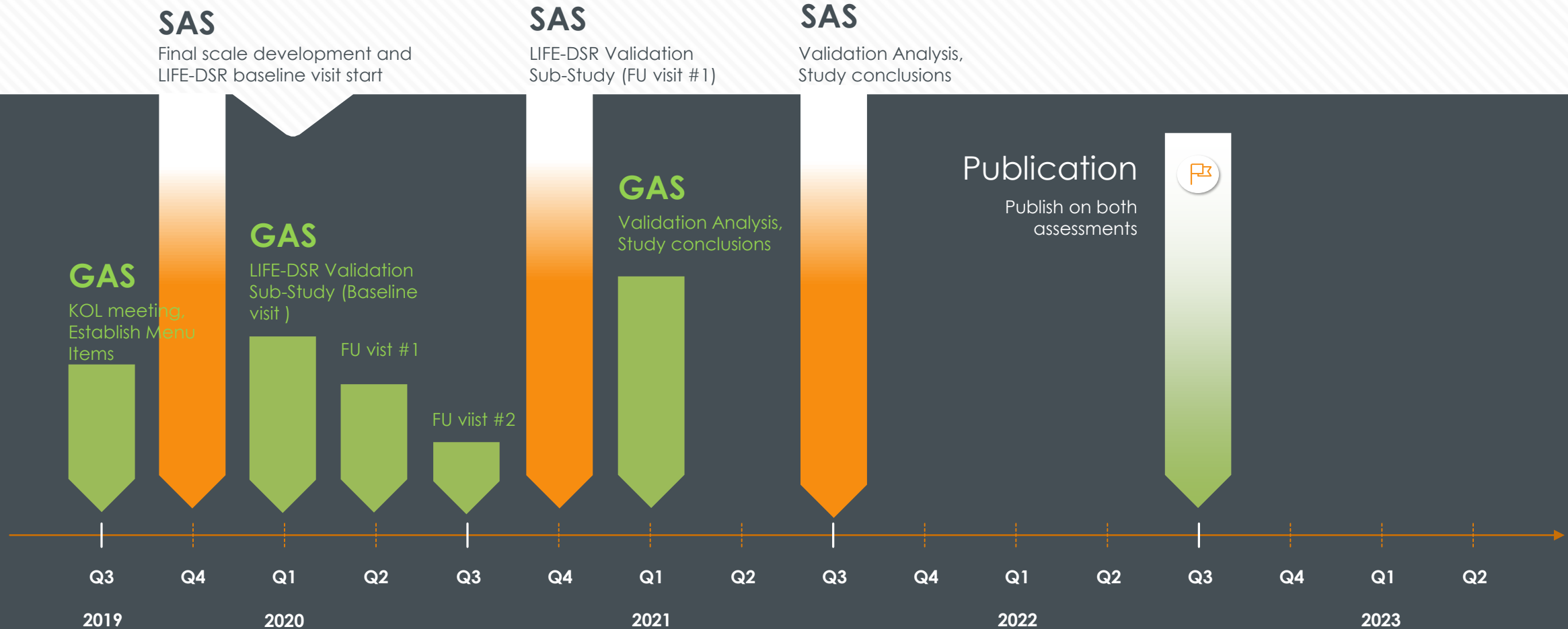
GAS IS CLINICALLY MEANINGFUL

- › 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

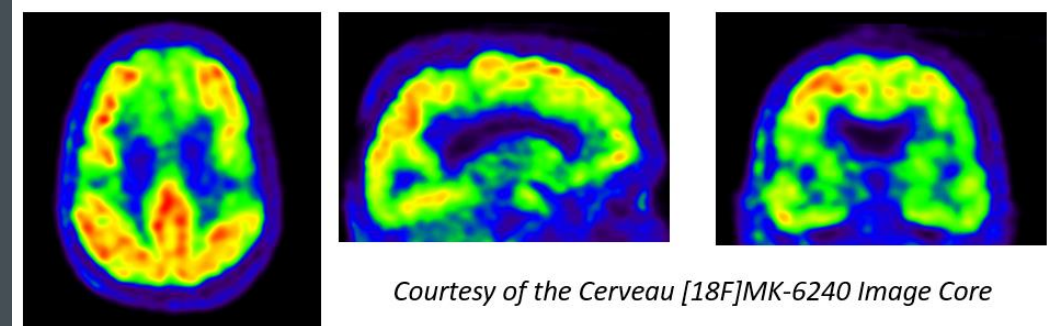
New Assessment Scales Timelines and Validation in LIFE-DSR



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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