Leading the way in Down syndrome research: Why we should be excited about the future

DSGKC Families Research Presentation, November 19, 2019
Hampus Hillerstrom, President and CEO
Dr. Jim Hendrix, Chief Scientific Officer

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

LuMind IDSC inspired and led by families like you!
Workshop Agenda

1. About medical advances and the status of research in Down syndrome
2. About LuMind IDSC Foundation
3. LuMind IDSC Strategic Focus Areas
4. Highlights on exciting research in Down syndrome
5. Rallying behind research – learning from the cystic fibrosis field
6. Take-home messages for families

1

About medical advances and the status of research in Down syndrome
Life expectancy and the number of people with Down syndrome has increased dramatically

Estimated Life Expectancy at birth over time:

<table>
<thead>
<tr>
<th>Decade</th>
<th>1950s</th>
<th>1980s</th>
<th>1990s</th>
<th>Today</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number</td>
<td>12</td>
<td>25</td>
<td>50</td>
<td>&gt;55</td>
</tr>
</tbody>
</table>

US Down syndrome population (1950-2013):

Source: Graaf et al., 2019

Medical treatments and educational support strongly benefit children and adults with Down syndrome...

Heart surgery
Ear Tubes
Treatments for thyroid disorder
Glasses
Meaningful Inclusion
CPAP Mask
Early Intervention
... however, several major medical needs remain in Down syndrome:

- Higher likelihood to develop certain forms of leukemia: 20x
- HAVE A FORM OF IMMUNE CONDITION: >50%
- HAVE SLEEP APNEA OR SLEEP DISORDERS: ~80%
- HAVE SPEECH DIFFICULTIES; >10% ARE NON-VERBAL: 100%
- WOULD BENEFIT FROM TREATMENTS TO IMPROVE INDEPENDENCE: 100%
- HAVE THE PATHOLOGY OF ALZHEIMER’S DISEASE BY AGE 40: 100%

There is a near-term pipeline of translatable, potentially impactful therapies across life span for Down syndrome:

<table>
<thead>
<tr>
<th>Approach</th>
<th>Potential Impact</th>
<th>Timing of therapy</th>
<th>Potential time to clinical trials</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hypoglossal stimulation</td>
<td>Sleep, speech, cognition,</td>
<td>Adolesc., adults</td>
<td>Ongoing</td>
</tr>
<tr>
<td>Infant learning</td>
<td>Independence, learning</td>
<td>Infants/toddlers</td>
<td>Ongoing</td>
</tr>
<tr>
<td>Amyloid beta targeting approaches</td>
<td>Independence, Alzheimer</td>
<td>Adults &gt;30 y.o.</td>
<td>Ongoing</td>
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<tr>
<td>Drug combination for sleep apnea</td>
<td>Sleep, cognition</td>
<td>Lifespan</td>
<td>2020</td>
</tr>
<tr>
<td>JAK inhibitors</td>
<td>Immune, skin, alopecia, other</td>
<td>Across life span</td>
<td>2020</td>
</tr>
<tr>
<td>Digital medicines</td>
<td>Independence, speech, exec. func.</td>
<td>Across life span</td>
<td>1+ years</td>
</tr>
<tr>
<td>Tau targeting approaches</td>
<td>Independence, Alzheimer</td>
<td>Adults &gt;30 y.o.</td>
<td>1+ years</td>
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<tr>
<td>Gamma wave device</td>
<td>Independence, Alzheimer’s</td>
<td>Adolesc., adults</td>
<td>1+ years</td>
</tr>
<tr>
<td>Dynk1a target</td>
<td>Independence, cognition</td>
<td>Early &amp; life span</td>
<td>3+ years</td>
</tr>
<tr>
<td>Myelination target</td>
<td>Independence, communication</td>
<td>Early to adulthood</td>
<td>3+ years</td>
</tr>
<tr>
<td>Gene therapies</td>
<td>Independence, Alzheimer, infection</td>
<td>Early &amp; life span</td>
<td>5+ years</td>
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About LuMind IDSC Foundation

Exciting news! LuMind RDS & IDSC merged on 3/21/19!

<table>
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<tr>
<th>Focused on research</th>
<th>Focused on engagement with DS community</th>
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<tbody>
<tr>
<td>Connected to top academic, industry and government DS scientists and 27k followers</td>
<td>Connected to 250K followers via Social Media (150K in US; 100K international)</td>
</tr>
<tr>
<td>Increasing research awareness through educational programs for families</td>
<td>Engages with families via social media support groups and events throughout the US</td>
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The combination will strengthen the connection between DS research and families who will benefit most from the research.
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).

LuMind IDSC research approach is focused on collaborative, translational DS research and has supported 16 clinical trials on >1300 participants to date.

LuMind IDSC has the largest Down syndrome community with 273,000 followers on Facebook, 16 Facebook support groups and an annual in-person event for 4400 family members.

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

The research that LuMind has funded has led to the discovery of 10 potential therapeutic targets and the development of 4 assessment scales for children and adults.

Our Leadership Team

Hampus Hillerstrom
President & CEO
- 10 years in biotech
- 4 years venture capital/investment banking
- MBA Harvard, MSc MIT/HMS, MSc Economics University St. Gallen
- Parent of 5 yo Oskar with Down syndrome

Dr. Jim Hendrix
Chief Scientific Officer
- 20 years in pharma/biotech
- 5 years at Alzheimer’s Association
- PhD Colorado State University

Michelle Petronio
Chief Development Officer
- 20+ years non-profit fundraising experience
- Career at DCA Consulting
- Managed large campaigns
- Master of Law and Social Policy and Master of Social Services from Bryn Mawr College, B.A. from University of Colorado, Boulder
- Parent of 14 yo Oliver with Down syndrome

Beth Sullivan
Sr. Dir. Community Engagement
- Co-founder/leader of IDSC, a 250k DS community
- Former freelance editor and writer on academic dissertations, theses, and curriculum development
- B. A. in journalism from Indiana State University
- Parent of 14 yo Oliver with Down syndrome
We accelerate research with a network of world-leading scientists, clinicians and drug developers.

**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.

**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
LuMind IDSC collaborated actively over the last two years for research, awareness and medical care.

We are thankful for the external endorsement of our efforts to rapidly advance our mission.

LuMind IDSC selected Finalist in Non-profit category
LuMind IDSC Awarded Pueschel-Tjossen Memorial Research Award
LuMind IDSC has 3 Strategic Focus Areas

1. **Research Awareness** - Engage the community on research
   - Families surveys
   - Facebook support group on research
   - Research webinars series
   - Clinical Trial Finder
   - Other research awareness efforts

2. **Accelerate Research** - Enable and advance research projects
   - DS-CTN clinical trial network
   - Assessment scales
   - Support specific research projects
   - Translational experts meetings
   - Engagement with industry and NIH

3. **Empower families** - Education, connections, and support
   - DSC2U collaboration with MGH
   - Facebook support groups
   - Great Wolf Lodge Week-end for families
   - Information and community news online
   - Photo campaign
## LuMind IDSC has 3 Strategic Focus Areas

<table>
<thead>
<tr>
<th></th>
<th>Research Awareness</th>
<th>Accelerate Research</th>
<th>Empower families</th>
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<tbody>
<tr>
<td>1</td>
<td>Engage the community on research</td>
<td>Enable and advance research projects</td>
<td>Education, connections, and support</td>
</tr>
</tbody>
</table>

### Research Awareness
- Families surveys
- Facebook support group on research
- Research webinars series
- Clinical Trial Finder on website
- Other research awareness efforts
- DS-CTN clinical trial network
- Assessment scales
- Support specific research projects
- Translational experts meetings
- Engagement with industry and NIH
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### Accelerate Research
- DS-CTN clinical trial network
- Assessment scales
- Support specific research projects
- Translational experts meetings
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### Empower families
- DS-CTN collaboration with MGH
- Facebook support groups
- Great Wolf Lodge Week-end for families
- Information and community news online
- Photo campaign

### Family survey on knowledge, attitudes, behaviors towards Down syndrome research

- Conducted by Antioch University PhD candidate Nicole White.
- LuMind IDSC worked with Dr. Nicole White as well as other individuals on the survey questions.
- IRB approved in February 2018.
- LuMind RDS and IDSC distributed the survey by email and Facebook.
- Responses from 256 parents of individuals with Down syndrome (publication pending).

A big thank you to the participating parents!
1. **Results from family survey on research**

   *Parent-reported needs (open-ended questions)*

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### Behaviors

- Sensory Skills
- Social Skills
- Independence
- Speech
- Cognition

### Abilities

- Motor Skills
- Communication
- Self Awareness
- Independence
- Academic

### Conditions

- Hypothyroidism
- Cognition
- Sleep Apnea
- Alzheimer's
- Communication

### Research Interests

- Speech Development
- Cognition
- Alzheimer's

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**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=159+, Spring 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
1 Results from family survey on research
Parent-reported concerns

Top 3 Concerns
1. Risks
2. Transparency
3. Intent

Do you currently have any concerns about enrolling your child/dependent with Down syndrome in a research study?

- Yes: 37%
- No: 30%
- Unsure: 33%

1 Results from family survey on research
Parent-reported wish for more research

Would you like to see research based on the latest scientific findings and cutting-edge technology to be conducted toward the improvement of health and independence for your child/dependent with Down syndrome?

- Yes: 98%
- No: 0%
- Other: 0%
Results from family survey on research

Parent-reported wish for drugs, therapies & interventions

Do you want to see more drugs, therapies, and interventions available for your child/dependent with Down syndrome in order to improve their health and independence?

- 86% Yes
- 0% No
- 0% I feel my current choices are adequate
- 0% Other

Inconsistent with 86% of parents wanting more drugs, therapies and interventions for their child/dependent with Down syndrome.

* NOT drugs, blood draws, imaging, implants, etc.

1. Distance to research study site
2. Access to Results
3. Clear Purpose
4. Low Invasive Procedures*

* NOT drugs, blood draws, imaging, implants, etc.
Results from family survey on research

Parent-reported participation in research studies

How many studies has your child/dependent with Down syndrome participated in?

- 64%
- 21%
- 7%
- 7%
- 0%
- 1%
- 2%
- 3%
- 4%
- 5%

To help our children/adults with DS and accelerate new treatment options it is critical to participate in research!

Results from family survey on research

Parent-reported resources for research knowledge

Top 3 Resources
1. Google
2. Non-Profit
3. Social Media

Only 54% feel they can separate “good” from “bad” information

Only 12% feel that there are enough resources for parents to know the status of research
In February 2019, we launched a Facebook support group for Research & Medical Care

- >1100 members in recently launched Facebook group
- Share articles on evidence-based research and medical care
- Host group-exclusive webinars to provide updates on the latest research endeavors;
- Engage with families and caregivers as well as answer your questions.
- Safe place to share, connect, and learn.
- Link to sign up: https://www.facebook.com/groups/LuMindIDSCResearch/

In collaboration with IDSC, we launched a Facebook support group for Research & Medical Care

- Discuss evidence-based research
- Engage with families
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

Visit the DS-CTN website: www.ds-ctn.org
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 in DS-CTN network.

- **Test/develop assessment scales for future clinical trials:**
  - Sensitive and well-validated instruments for cognition, behavior, function and health
  - Includes two new scales: Strydom Scale and Goal Attainment scales

- **Longitudinal data collection:**
  - Physical exam, medical history, cognitive, behavioral and functional testing, and blood draw
  - 3 clinic visits, over 24 months

LIFE-DSR – Who will be in the study?

- 270 participants
- Age 25 and older
- 80% between the ages of 35-55
- Very few exclusion criteria
  - Only people that, in the opinion of the investigators, can’t complete the study requirements will be excluded.
2 LIFE-DSR Kick-off meeting with the 11 clinics
March 13, 2019 – Bethesda, MD

2 LIFE-DSR Status – Nov. 2019

**LIFE-DSR Site Activation**
- 9 Sites Approved to open recruitment (1st in July)
- Activation of 3 sites still in progress – should open in Nov/Dec
- LuMind IDSC to support recruitment with local press releases and social media posts
- Study governance in place

<table>
<thead>
<tr>
<th>Active Sites (PI)</th>
<th>Subjects Baselined</th>
</tr>
</thead>
<tbody>
<tr>
<td>MGH 1 (Skotko)</td>
<td>13</td>
</tr>
<tr>
<td>MGH 2 (Lai)</td>
<td>1</td>
</tr>
<tr>
<td>Barrow (Burke)</td>
<td>1</td>
</tr>
<tr>
<td>Advocate (Chicoine)</td>
<td>17</td>
</tr>
<tr>
<td>Duke (Kishnani)</td>
<td>8</td>
</tr>
<tr>
<td>Emory (Sherman)</td>
<td>2</td>
</tr>
<tr>
<td>Rush (Ochoa-Lubinoff)</td>
<td>1</td>
</tr>
<tr>
<td>Cincinnati Children’s (Eibensen)</td>
<td>0</td>
</tr>
<tr>
<td>Case Western (Costa)</td>
<td>0</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>43</strong></td>
</tr>
</tbody>
</table>
New Assessment Scales Timelines and Validation in LIFE-DSR

Note: SAS: Strydom Assessment Scale; GAS: Goal Attainment Scale; GAS timeline is pending additional funding for validation part.

Tau PET imaging Collaboration in LIFE-DSR

- Collaboration announced with Cerveau Technologies on May 9th
- 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.
- Planning to submit NIH grant and/or secure industry funding
3 Launching DSC2U in 2020 in collaboration with MGH

Down Syndrome Clinic to You (DSC2U)
An online personal care plan for caregivers and primary care physicians

Brian Skotko, MD, MPP
Director, Down Syndrome Program
Massachusetts General Hospital

- Medical information platform to help develop a plan for your medical care with your primary care physician
- Addresses a major need for the 95% of individuals with DS who do not have access to a specialized DS clinic
- LuMind IDSC is the founding philanthropic supporter and collaborates with MGH to provide access through our 273,000 online followership
- Launching in 2020. Stay tuned!

www.dsc2u.org

3 LuMind IDSC empowers the Down syndrome community through social media!

- Community of 273,000 on Facebook
- 16 Facebook support/focus groups with > 15,000 members total
- Groups range from ages and stages of life, to specific topics

<table>
<thead>
<tr>
<th>Adoptive Family Support</th>
<th>Babies and Toddlers</th>
<th>Connections</th>
<th>Conexion en Espanol</th>
</tr>
</thead>
<tbody>
<tr>
<td>161 members</td>
<td>3,410 members</td>
<td>4,048 members</td>
<td>398 members</td>
</tr>
<tr>
<td>Dads</td>
<td>309 members</td>
<td>Educators</td>
<td>Friends and Family</td>
</tr>
<tr>
<td>370 members</td>
<td></td>
<td>215 members</td>
<td>398 members</td>
</tr>
<tr>
<td>Grandparents*</td>
<td>Love and Loss</td>
<td>Pre-K and Elementary</td>
<td>Preteens/Teens/Adults*</td>
</tr>
<tr>
<td>347 members</td>
<td>109 members</td>
<td>1,640 members</td>
<td>2,135 members</td>
</tr>
<tr>
<td>Prenatal Diagnosis</td>
<td>Research &amp; Medical Care</td>
<td>Self-Advocates</td>
<td>Siblings</td>
</tr>
<tr>
<td>609 members</td>
<td>&gt;1,100 members</td>
<td>71 members</td>
<td>346 members</td>
</tr>
</tbody>
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LuMind IDSC Family Week-End @ Great Wolf Lodge offers an opportunity to connect locally

17 Locations
The LuMind IDSC Family Weekend at Great Wolf Lodge began in 2013 at 5 locations across the United States. Since then, it has grown to 17 locations, 16 in the United States and 1 in Canada. Our event grows as Great Wolf Lodge expands and opens new locations.

4,400+ Participants
Over 920 families are expected to join us this year.

And growing …
We’ve seen major growth 2013-2019. Our event also grows as Great Wolf Lodge expands and opens new locations. We anticipate the 18th and 19th locations in Arizona and California in 2020.

Join us at your local GWL on the week-end of September 18-20, 2020! SAVE THE DATE!

Highlights on exciting research in Down syndrome
Highlighted exciting research areas in Down syndrome

A. Major momentum on cohorts and networks
B. Promising drug candidates for Alzheimer's
C. Digital medicines for cognition, behavior and speech
D. Gene or chromosome silencing for cognition, Alzheimer's and auto-immune conditions
E. Interferon-targeted treatments for auto-immune conditions
F. Hypoglossal stimulation for sleep apnea, speech and cognition

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
A. International 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
- Human Trisome Project cohort: 520 cohort in US
- LIFE-DSR cohort: 270 cohort in US
- NIH ACTC-DS network: Emerging network
- DS Cognition Project cohort: ~300 cohort
- Horizon21 network: 7 sites in EU

A. Exciting new approach for cognition in Down syndrome!

In Down Syndrome Mouse Model, Scientists Reverse Intellectual Deficits with Drugs

In a surprising finding using the standard animal model of Down syndrome (DS), scientists were able to correct the learning and memory deficits associated with the condition — the leading genetic cause of cognitive disability and the most frequently diagnosed chromosomal disorder in the U.S. — with drugs that target the body’s response to stress.
B. LuMind RDS 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


EPIDEMIC

CRISIS!
B. Promising progress towards treating Alzheimer’s – Aducanumab!

- Oct 2019: Biogen announced in that they will seek FDA approval in Alzheimer’s for the general population.
- Unexpected turn of events based on a re-analysis of all Phase 3 data available
- Opens up potential for treating Alzheimer’s in Down syndrome

B. Promising late-stage Alzheimer’s molecules will require testing in adults with Down syndrome

**TARGETING AMYLOID BETA**
- Lilly: Donanemab
- Biogen / Eisai: BAN2401
- Biogen / Eisai: Aducanumab

**TARGETING TAU**
- Biogen: Gusuranemab
- Biogen: BIB076
- Biogen / IONIS: BIB080
- Abbvie: ABBV-8512
- Lilly: LY3303560 (Gagogenemab)
- Roche: RG100

Source: clinicaltrials.gov; press releases
C. Digital medicine for Down syndrome
The Advances

Improved brain imaging techniques and better understanding of brain neurocircuitries have enabled the development of Digital Medicines focused on improving certain brain neurocircuitries.

Source: Akili Interactive website (Feb 2018)

C. Digital medicine for Down syndrome
What it looks like

Digital Medicine is delivered through a tablet game targeting key functional cognitive networks in neurological processes relevant to certain conditions.
C. Digital medicine for Down syndrome
Where the field is and how it could be applied to Ds

Current applications and progress:

How could it be applied in Down syndrome:
In Down Syndrome a Digital Medicine could target initially cognitive functions such as attention deficits, superior executive function, working memory and other functions related to cognitive control.

D. Gene or chromosome silencing on extra Chr 21
What is the concept

General concept based on a natural phenomenon:
- Restore the balance: for a specific gene of concern, a region that includes several genes, or the whole chromosome.
- XIST gene: naturally active gene that leads to the silencing of one of the two copies of Chromosome X in women.

How it can be applied to Down syndrome:
- Use gene therapy to deliver XIST gene to Chromosome 21;
- Promising, but several technical steps need to be worked out;
- Exciting opportunity: gene therapy to silence the Alzheimer's APP gene (triplicated on Chromosome 21) or balance the immune system.
D. Progress and growing interest in gene/chromosome therapy for Down syndrome

Recent results on the applicability for immune conditions

E. Chronic state of inflammation in Down syndrome

Promising new research avenue

- 4 inflammation genes (interferon receptor genes) are “triplicated” on Chromosome 21
- Researchers have shown that these genes can be connected to the observed chronic state of inflammation (multiple papers since 2016).
- Promising applications in Down syndrome: auto-immune (such as alopecia areata “spot baldness”) and immune-related conditions.
- Near term applicability:
  - JAK inhibitors drugs in development or approved for rheumatoid arthritis and other conditions.
  - Clinical trial with JAK inhibitor for alopecia areata in planning (NIH INCLUDE grant awarded).
F. Hypoglossal Stimulation for Sleep Apnea in Ds

- Implant a device to stimulate the tongue to open airway at night
- Ongoing 50 participant trial at 10 sites (4 active)

- **Exciting interim results (data on 21 patients):**
  - 84% reduction in sleep apnea index
  - 7.9 hour/night compliance (compared to ~3 hours for CPAP mask alternative).

- LuMind RDS is working with principal investigator Dr. Chris Hartnick (Mass Eye & Ear) to accelerate the availability of this approach for children with Down syndrome.
  - Data collection
  - NIH application
  - Interaction with study sponsor

F. Hypoglossal stimulation also showed anecdotal reports of improvement of speech/cognition

- “The smoothness of conversation has gained since the surgery but the most gain has been with articulation. Everything has improved!”

- “He will be a senior in high school next year, he never ever would have made it without hypoglossal stimulation and sleep, his speech has improved enough he can be understood in school.”

- “The IEP showed increase fluency in speech, more organized in thoughts and organization. He showed an ability to self monitor speech and to regulate his speech. He was able to pay attention and focus in class. He showed an increase from previous evaluation.”

LuMind RDS funds the collection of speech/cognition data in 10 participants in ongoing clinical trial
Rallying behind research – learning from the cystic fibrosis field

When people ask me to provide an example of how patients, caregivers, researchers, a Foundation, NIH and industry can all work together to find cures, I point to cystic fibrosis. It's the very best example.

In the last 15 years, a rallied Cystic Fibrosis (CF) community led to >125 clinical trials and 5+ drug approvals.
How does Down syndrome compares to Cystic Fibrosis?

<table>
<thead>
<tr>
<th>Year</th>
<th>Cystic Fibrosis</th>
<th>Down syndrome</th>
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<tbody>
<tr>
<td>Research-focused organizations</td>
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<tr>
<td>Promising science and therapeutic targets</td>
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<tr>
<td>Attendance research meetings</td>
<td>☺☺☺</td>
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<tr>
<td>Research analyst tools</td>
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<tr>
<td>Gene therapy field</td>
<td>☺</td>
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<tr>
<td>NIH funding</td>
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<tr>
<td>Industry interest</td>
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<td>☺</td>
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<tr>
<td>Clinical trial network</td>
<td>☺</td>
<td>☺</td>
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<tr>
<td>Knowledge of community on research</td>
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<td>Rallying community behind research</td>
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<td>Research-focused organizations funding</td>
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KEY AREAS OF NEED TO ADVANCE NEW TREATMENT OPTIONS FOR DOWN SYNDROME

What is LuMind RDS doing to Rally the Down syndrome Community?

1. Presentations to inform about status and promise of Down syndrome research
2. Research webinars to inform and seek engagement on topics of research
3. Survey on “Knowledge, attitude and behavior towards Down syndrome research”
4. Collaborate with other Down syndrome organizations
5. Engage with the NIH to support more research
6. Engage with industry to support more research
7. Recent: Clinical Trial Finder on LuMind IDSC website
8. Recent: Facebook support group on Research and Medical Care
9. Recent: IDSC and LuMind RDS merged
TOGETHER as a community we can move mountains!

6
Take-home messages for you
4 Ways you can Rally around Research!

1. Get educated - on the current status of Down syndrome research and stay connected
2. Engage on research - with other families, LuMind RDS, researchers and clinicians
3. Participate - in research studies and clinical trials
4. Give - time or donation, or help fundraise for research
Use our Research Webinars resource to get up to speed on latest research topics

You can access the LuMind RDS educational webinars here: https://www.lumindrds.org/learn/webinars-and-presentations/

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Your families can also sign up to the FB Group on Research and Medical Care

Sign up here to this Facebook support group: https://www.facebook.com/groups/LuMindIDSCresearch/

>1,100 members as of November 2019

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There are 6 research studies in Kansas that your family member with Down syndrome can participate in

1. A Study to Determine the Outcomes of Patients With Localized B Cell Lymphoblastic Lymphoma (B-LLy) When Treated With Standard Risk B-ALL Therapy [1-31 years]
2. Promotion of Physical Activity in Adolescents With Intellectual and Developmental Disabilities [10-21 years]
4. Study of Blood Samples From Newborns With Down Syndrome [0-90 days]
5. Weight Management for Adolescents With IDD (IDDAdol) [13-21 years]
6. Response-Based Chemotherapy in Treating Newly Diagnosed Acute Myeloid Leukemia or Myelodysplastic Syndrome in Younger Patients With Down Syndrome [91 days – 3 years]

And many research surveys! Stay tuned for LuMind IDSC’s independence survey (Nov 2019).

You can monitor ongoing studies through the LuMind IDSC Clinical Trial Finder

[Link on LuMind IDSC website: https://www.lumindrds.org/clinical-trial-finder/]
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A self-advocate’s call for Rallying behind Research (Karen Gaffney)
Thank you!

Visit us at: www.LuMindIDSC.org

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