RESEARCH AWARENESS *community*

MAY 2022 – Inaugural Quarterly Meeting

May 2022 Agenda

- **Welcome & Introductions**
  - **Context info: Why are we doing this?**
  - **Overview of the goal and scope of this group**

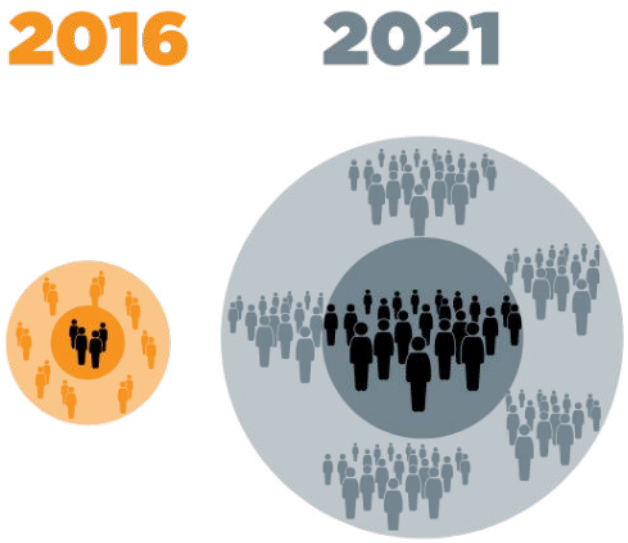
- **Research Toolbox Topic: Vetting research requests**
  - **How to respond to a request from a researcher**
  - **Checklist for vetting requests**

- **Community Voice**
  - **Your Questions on Research** – what is your community talking about? Share topics of concern.
  - **How can we help?** – what information or resources do you need?
  - **Your suggestions for next steps**

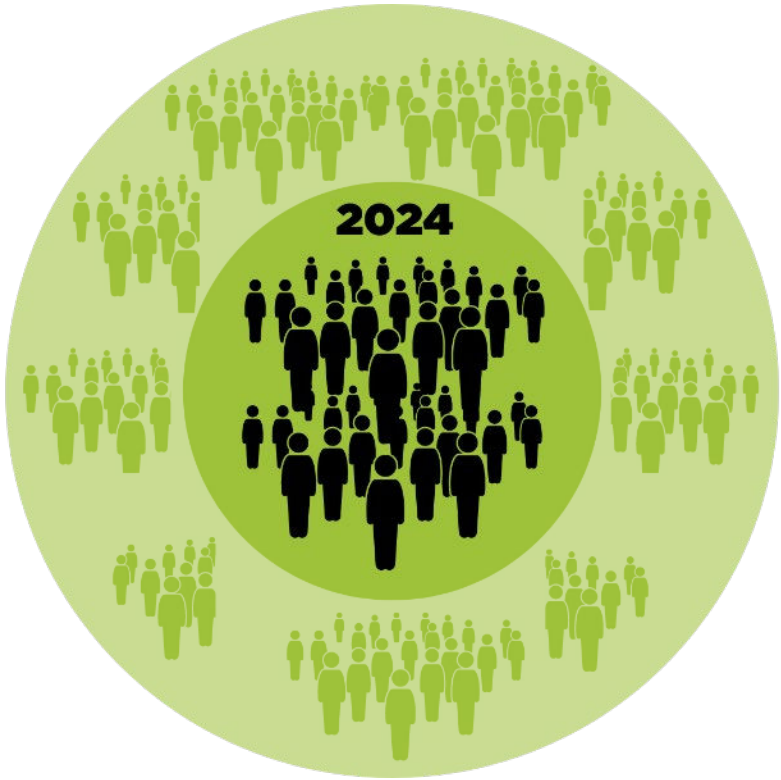
- **Get Technical: An update on latest DS-CTN/Research Consortium initiatives:**
  - **Goal Attainment Scale – first of its kind assessment scale for DS-AD tasks of daily living**
  - **Biomarker Collection Sub-studies –first instances of MRI, PET scan, and CSF collection**

Trial participation is critical

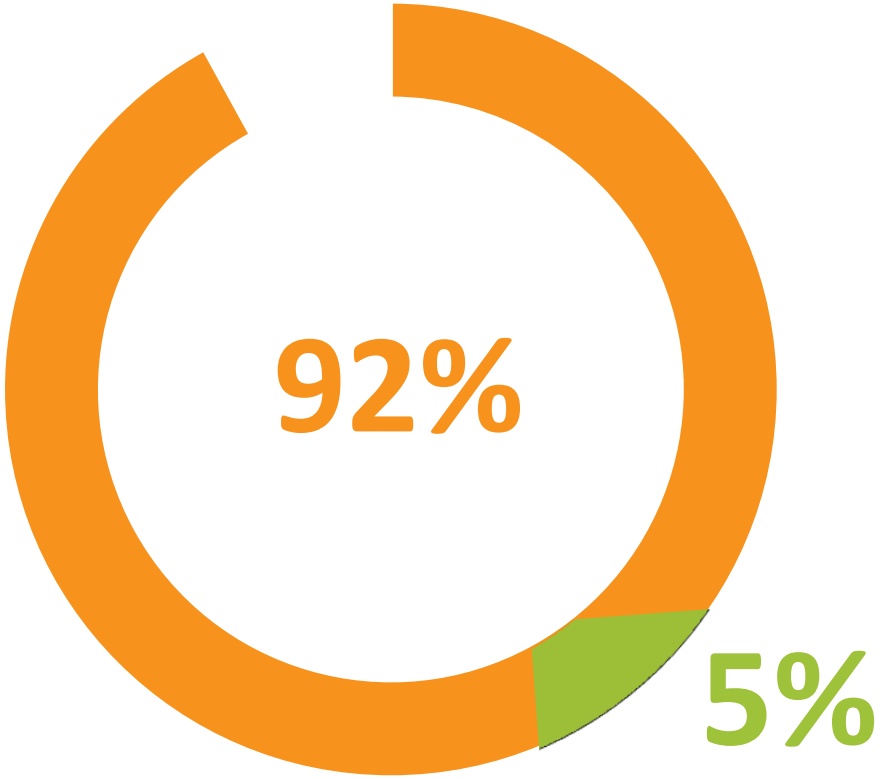
The number of clinical trials and observational studies focused on adults with Down syndrome grew from one trial in 2016 to four in 2021.



5-10 studies are expected by **2024**.



Research Participation Disparity



92% of families surveyed by LuMind IDSC indicated they wanted clinical trials to focus on the health care needs of people with Down syndrome.

Fewer than **5%** of families said they had ever participated in a clinical trial.

CONCLUSION: We need to raise awareness of research opportunities, and de-mystify research for our families.

Families remain skeptical toward participation

Results from survey on 370 caregivers of adults LuMind IDSC, NDSS, and principal investigator Dr. Eric Rubenstein (manuscript in process)

- Observational study (66% would participate in a trial)
- Exercise & diet studies (54% to 63%)
- Laboratory exams like MRIs (32%)
- Interventional Clinical trial (4% to 25%, depending on frequency of administration and stage of drug)

LuMind IDSC is uniquely positioned to Accelerate Research

The Down Syndrome Clinical Trial Network (**DS-CTN**) now includes 14 clinical sites in 10 states.

We currently **collaborate with 15 pharma**, biotech, medical device, digital health, and diagnostic companies.

Led by a Research Team of six, our robust program has involved **1500 trial participants** and **7900 survey respondents** to date.

Recently led a collaboration of **50+ experts** for work with the National Institutes of Health (NIH).

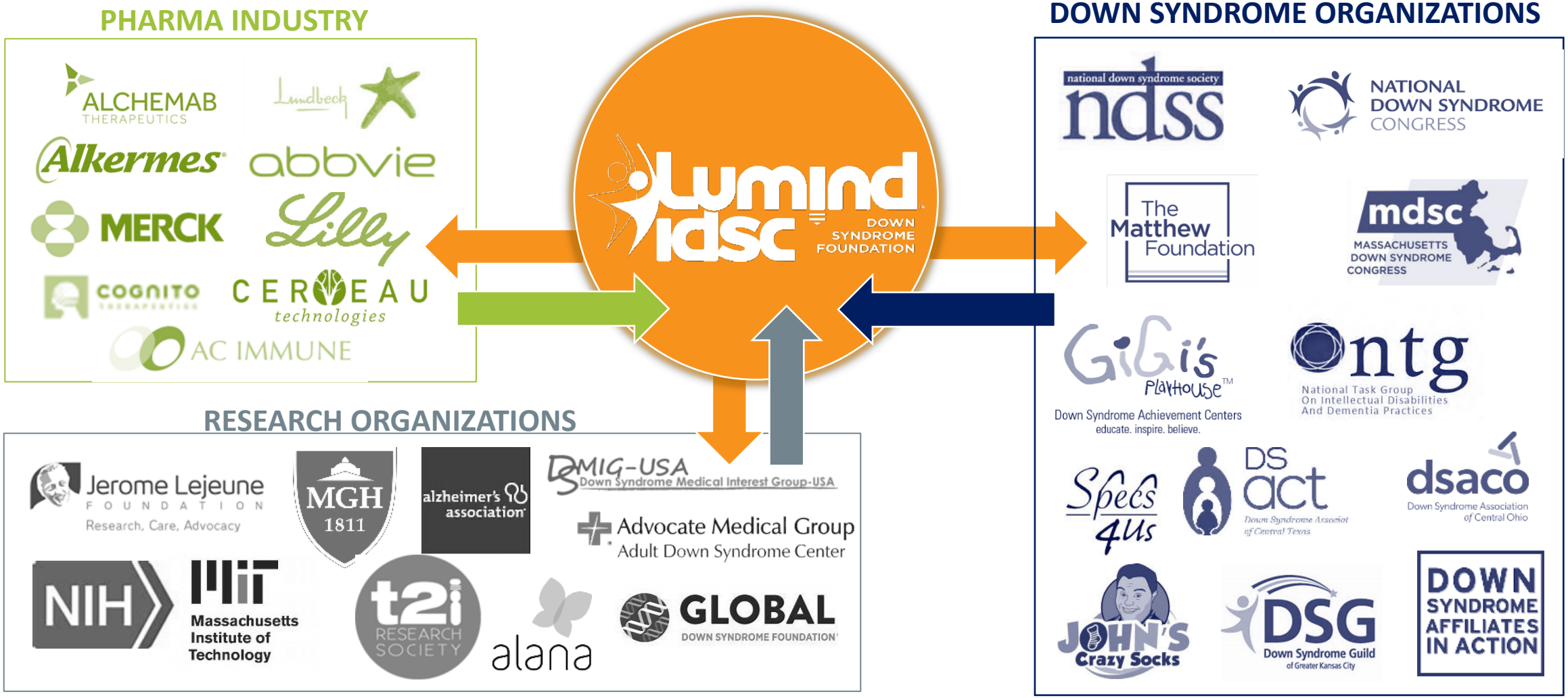
Launched the **LuMind IDSC Down Syndrome Research Consortium** with key support from Merck & AbbVie.

Led a **Critical Path Innovation Meeting** (CPIM) with the U.S **FDA** in 2021 to bring industry & government attention to the barriers to DS-AD research.

Awarded **140 research grants**, supported **21 observational and interventional clinical studies**, developed **4 cognitive tests** and Alzheimer's assessment scales.

Supported multitude of **translational research projects** in Alzheimer's, sleep apnea, speech, and independence.

Collaborating across all communities, moving forward together



Research Awareness & Info IN THE COMMUNITY

An as-yet un-named alliance of community-based Down syndrome and other organizations informing and promoting research awareness

Our vision for research awareness & participation in 2022-2024

- Fact-finding conversations with leaders of community-based organizations to inform a framework for collaboration/consortium/alliance
- Formalize LuMind IDSC partnerships with Down syndrome organizations, and expand to include IDD non-profit collaborators
- Help non-profit collaborators solve research-related problems, share best practices, and access latest research information
- Offer a pathway for partners to discuss research topics with LuMind IDSC and contribute to research strategy priorities
- Establish a two-way amplification system for LuMind IDSC awareness initiatives and non-profit collaborators' opportunities

Our goal: significantly increase research awareness and participation in the Down syndrome community

Our partner benefits:

- **It's free:** No membership fee for community partners who join LuMind IDSC's formal research awareness collaboration
- **Participation in quarterly forum:** Speak up, help identify the research needs of the DS community. Use opportunity to bring concerns directly to research strategy architects and study designers
- **Take advantage of our expertise:** We're here to help organizations sort through requests for research participation/amplification and develop tools for vetting research requests and materials.
- **Be part of building a new community:** We anticipate growing this program over the next 2 years, and we want the scope and agenda to be directed by community input
- **Turn-key Research Awareness Materials:** Full access to library of research-related, family-facing materials, co-branded on request.
- **Latest Info:** Consortium "insider" explainers on latest in research news: DS-AD, LIFE-DSR, Sleep apnea, surveys, publications, and studies
- **Program referral:** your local/digital programs and events highlighted on LuMindIDSC.org and our social media channels

Partner deliverables to LuMind IDSC:

- **Participate:** Attend quarterly forum meetings, volunteer to lead discussions
- **Point of contact:** Identify one organizational point of contact who will work directly with LuMind IDSC on research-related communications, forum content, and initiatives
- **Amplify:** Share research information, research opportunities and initiatives, LuMind IDSC research-related materials, and communications with your community
- **Give feedback:** Provide input on key communications and initiatives and help shape future forums and initiatives
- **Partner:** Maintain communication with local DS-CTN site(s) as appropriate
- **Turn to us:** Prioritize LuMind IDSC as a trusted partner in research-related initiatives

Discussion:
How should we structure?

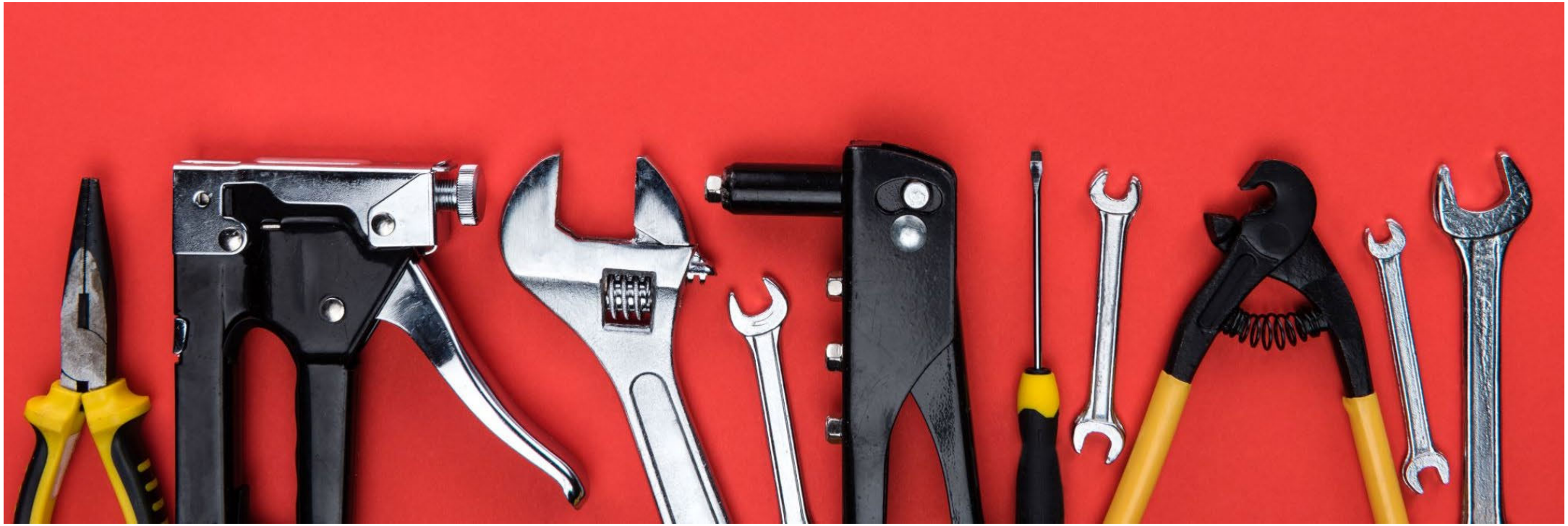
We need your input on channels of communication, “lift,” and community need/interest

What do organizations need right now, with regard to DS research?

What can LuMind IDSC do to help you address day-to-day research issues? Can we help you:

- Respond to research requests (access to your community)?
- Establish connections with clinics, specialists?
- Identify needed clinical/research resources through myDSC ?
- Prepare, draft explainers for the community – resources, guides, tips?
- Amplify your org’s research-related initiatives? Bring them to national audience?

RESEARCH TOOLBOX
for Affiliate Leaders



Tips for leaders responding to requests for your organization to participate in research studies, trials, or surveys

Responding to Research Requests

Decide beforehand how your organization can/should respond to requests

- Develop an internal process and basic guidelines
- Decide what type and level of involvement would work for your organization

There are many ways to support a research project

- Recruit participants for research studies, trials, surveys by sharing study information on your social media channels, in your newsletters, at your events, etc.
- Distribute a lay-language summary of a study/trial/survey and share the study impact, study findings, through reports to your community
- Influence a study/survey before it happens by representing your community and experiences on a study design committee, or by informally discussing DS and family-related priorities with a researcher
- Refer scientists to other Down syndrome-related experts in the field via your network
- Include the topic of research (general or study specific) in your local events

Responding to Research Requests

First steps when responding to an inquiry:

1. **Make an initial response.** Don't wait to respond. It is good to reply to an inquiry with: "This message has been received and we're considering it…" so that the researcher doesn't continue to follow up with additional inquiries.
   See packet for suggested language for initial response to inquiries

2. **Ensure the researcher has provided sufficient information** for you to evaluate their request.

3. **Evaluate the request** using one of the suggested **Vetting Checklists** or create your own that suits your community. Build a process that is efficient and easy for you. For example, create an online form or survey link that automates your checklist and automatically alerts you to a new request.

Responding to Research Requests

What is “Sufficient Information”?

Each org may have their own requirements, but we suggest the minimum information to evaluate a request includes:

- Contact information and credentials of the requestor or the individual who will be the main point of contact for questions
- A thorough explanation/description of the study/trial/survey. This may be through a protocol summary or more detailed information sharing.
- Proof of regulatory oversight (IRB, ERB, etc.) and approval of any materials they ask you to distribute, including images, outreach language, and social media language
- Outline of participant inclusion parameters
- Timeline components of the request (project anticipated start or end date), and
- Any other relevant information

Responding to Research Requests

OVERALL PRINCIPLES TO CONSIDER WHEN REVIEWING A REQUEST:

- Is the researcher connected to a respected academic, government, or healthcare institution?
- Are there other sponsors/partners associated with this study whose mission does not align with yours?
- Are the risks for participation clearly iterated (in family-centered language) and are the risks minimal?
- Do the inclusion parameters align with your community profile (if you serve mainly adults and the study is pediatric, etc.)?
- Is there an easy way for interested individuals to contact the study and receive a real-time response?
- Were people with Down syndrome and/or their caregivers included in the study design? If not, was expertise from the DS clinical community included?

Responding to Research Requests

OVERALL PRINCIPLES TO CONSIDER WHEN REVIEWING A REQUEST:

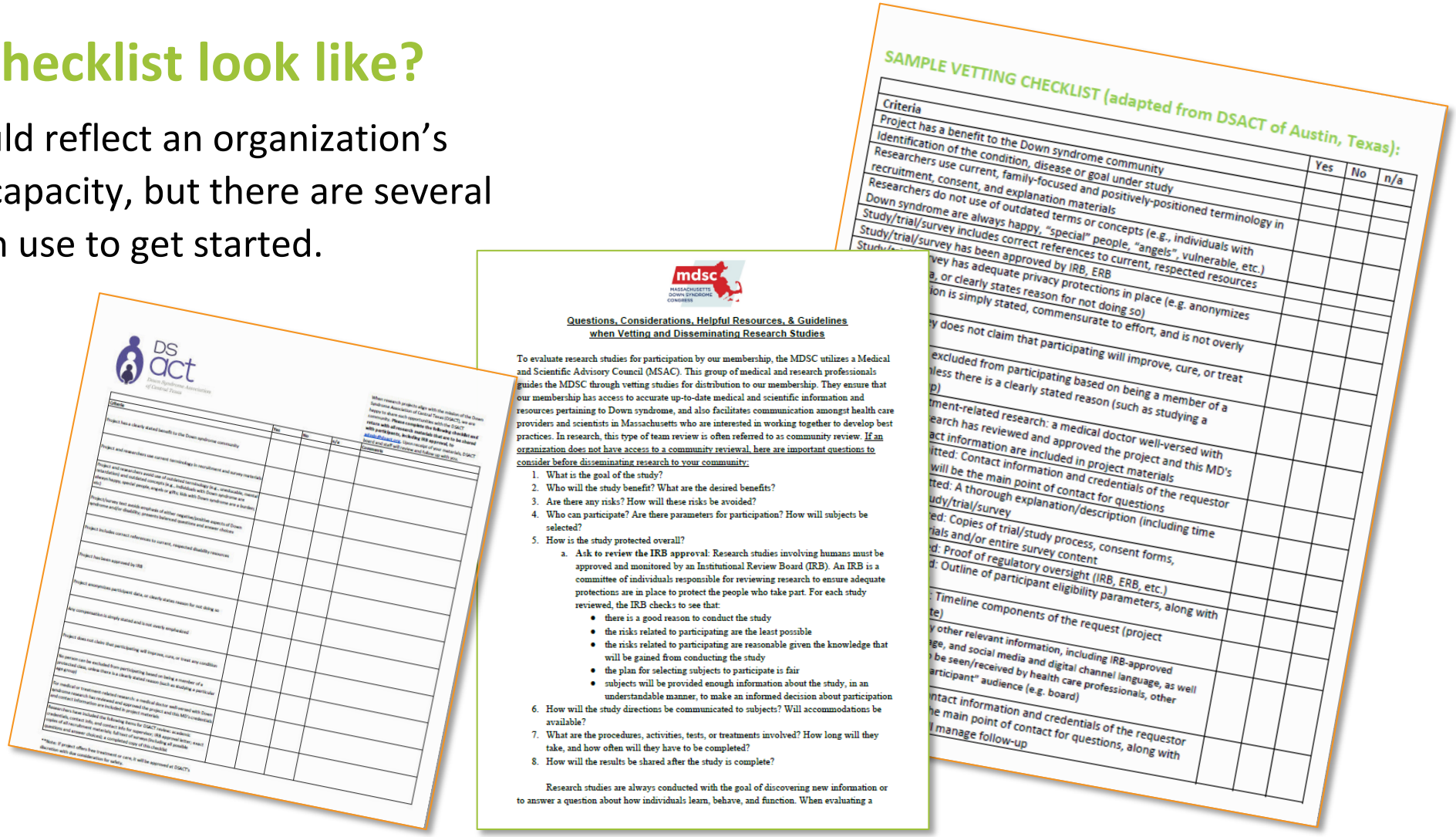
- Is the language around the study/trial/survey factual and not sensational? Does the study promise anything outrageous or improbable?
- Are the study materials and outreach collateral written with Down syndrome language that aligns to your community values?
- Is participant information de-identified and aggregated? If not, what is the reasoning?
- Is compensation for participation commensurate with the time invested?
- Is compensation directly benefitting the participant?

Responding to Research Requests

What would a Checklist look like?

Again, a Checklist should reflect an organization's values, interests, and capacity, but there are several guidelines that you can use to get started.

Packet contains sample checklist, and resource info for you to develop your own checklist.



SAMPLE VETTING CHECKLIST (adapted from DSACT of Austin, Texas):

Criteria	Yes	No	n/a
Project has a benefit to the Down syndrome community			
Identification of the condition, disease or goal under study			
Researchers use current, family-focused and positively-positioned terminology in recruitment, consent, and explanation materials			
Researchers do not use of outdated terms or concepts (e.g., individuals with Down syndrome are always happy, "special" people, "angels", vulnerable, etc.)			
Study/trial/survey includes correct references to current, respected resources			
Study/trial/survey has been approved by IRB, ERB			
vey has adequate privacy protections in place (e.g. anonymizes , or clearly states reason for not doing so)			
ion is simply stated, commensurate to effort, and is not overly			
y does not claim that participating will improve, cure, or treat			
excluded from participating based on being a member of a less there is a clearly stated reason (such as studying a p)			
tment-related research: a medical doctor well-versed with earch has reviewed and approved the project and this MD's act information are included in project materials			
itted: Contact information and credentials of the requestor will be the main point of contact for questions			
tted: A thorough explanation/description (including time udy/trial/survey			
ed: Copies of trial/study process, consent forms, rials and/or entire survey content			
d: Proof of regulatory oversight (IRB, ERB, etc.)			
d: Outline of participant eligibility parameters, along with			
: Timeline components of the request (project te)			
y other relevant information, including IRB-approved ge, and social media and digital channel language, as well be seen/received by health care professionals, other articipant" audience (e.g. board)			
ntact information and credentials of the requestor he main point of contact for questions, along with l manage follow-up			

Questions, Considerations, Helpful Resources, & Guidelines when Vetting and Disseminating Research Studies

To evaluate research studies for participation by our membership, the MDSC utilizes a Medical and Scientific Advisory Council (MSAC). This group of medical and research professionals guides the MDSC through vetting studies for distribution to our membership. They ensure that our membership has access to accurate up-to-date medical and scientific information and resources pertaining to Down syndrome, and also facilitates communication amongst health care providers and scientists in Massachusetts who are interested in working together to develop best practices. In research, this type of team review is often referred to as community review. If an organization does not have access to a community reviewal, here are important questions to consider before disseminating research to your community:

1. What is the goal of the study?
2. Who will the study benefit? What are the desired benefits?
3. Are there any risks? How will these risks be avoided?
4. Who can participate? Are there parameters for participation? How will subjects be selected?
5. How is the study protected overall?
   a. **Ask to review the IRB approval**: Research studies involving humans must be approved and monitored by an Institutional Review Board (IRB). An IRB is a committee of individuals responsible for reviewing research to ensure adequate protections are in place to protect the people who take part. For each study reviewed, the IRB checks to see that:
      - there is a good reason to conduct the study
      - the risks related to participating are the least possible
      - the risks related to participating are reasonable given the knowledge that will be gained from conducting the study
      - the plan for selecting subjects to participate is fair
      - subjects will be provided enough information about the study, in an understandable manner, to make an informed decision about participation
6. How will the study directions be communicated to subjects? Will accommodations be available?
7. What are the procedures, activities, tests, or treatments involved? How long will they take, and how often will they have to be completed?
8. How will the results be shared after the study is complete?

Research studies are always conducted with the goal of discovering new information or to answer a question about how individuals learn, behave, and function. When evaluating a

Responding to Research Requests

Accept, postpone, or reject a Research Relationship Request

Don’t hesitate to:

- Ask the researcher for more information if you don’t feel you can make a decision
- Reject a request and retain a relationship for future potential projects
- Move forward with confidence that you’ve vetted the request

Packet contains communication strategies for all of these scenarios

Responding to Research Requests

OTHER ASPECTS TO CONSIDER:

- Preparing your community to be “research ready” is an important step in the process. Bringing research-related language, facts, and materials into your organizational culture can help raise awareness and increase readiness for participation. LuMind IDSC has a ***suite of Research Awareness resources*** that are available to all affiliates for co-branding and broadcast.
- Some organizations establish ***permanent or ad hoc advisory councils*** to address research-related issues. The Massachusetts Down Syndrome Congress (MDSC) offered to share their materials and process with any organizations seeking to formalize their approach.
- Aligning your organization with ***local clinical partners*** is also a good option toward sharing the decision-making burden. Having a network of trusted clinical and research partners is a good way to build knowledge.
- Larger organizations may have the resources to ***add a part-time or full-time staff member*** to vet and coordinate community-facing research activities. There are several job descriptions that can be shared if your organization is looking to that avenue.

Special thanks to our collaborators

DS act
Down Syndrome Association of Central Texas

MASSACHUSETTS mdsc DOWN SYNDROME CONGRESS

for sharing their vetting resources!

COMMUNITY VOICES

Discussion: Your research concerns

Your Questions on Research – what is your community talking about? Share topics of interest or concern.

How can we help? – what information or resources do you need?

Discussion: Next steps

Two-way street communication plan

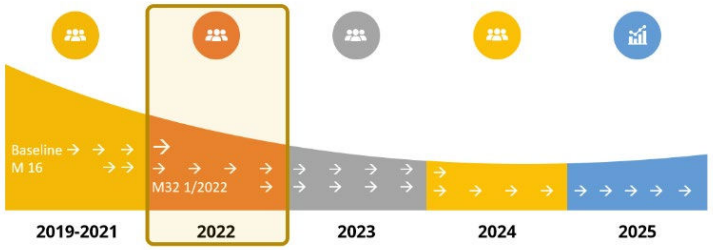
- Follow up email with summary of today's conversation and materials
- Please respond with your interest in "joining" the group
- Vote on a group name, and send suggestions for next quarterly meeting "Research Toolbox" topic
- Access ongoing info via http://www.lumindidsc.org/affiliates
- Send us your info and inquiries:
  - Hampus Hillerstrom (hhillerstrom@lumindidsc.org)
  - Kate O'Neill (koneill@lumindidsc.org)
  - Angela Britton (abritton@lumindidsc.org)

RESEARCH UPDATE: LIFE-DSR & sub-studies

LIFE-DSR Study

Longitudinal Investigation for the Enhancement of Down Syndrome Research

- **Observational, multi-center, longitudinal cohort study to characterize adults with DS ages 25 years and above, enrolled at specialized care centers.**
- **Aim**: assess changes in cognition, behavior, function and health over approximately 32 months.
- **Population**: 270 males & females with DS, 25 years and older
- **Visits**: Consists of 3 visits - Baseline, 16 months (+/- 2 months), and 32 months (+/- 2 months)
- **Data**: demographics, clinical, cognitive, behavioral, functional, blood collection
- **Participating sites**: 14 DS-CTN sites participating across 10 states within the U.S.
- **Target end of enrollment:** 2022
- **Target end of study:** December 2025



Participating Sites

LIFE-DSR Inclusion Criteria

1. **Age 25 years or older**
2. **Diagnosis of DS:** typically supported by karyotype analysis documenting full trisomy for chromosome 21 or complete unbalanced translocation of chromosome 21. *Karyotype analysis is not required for study entry.*
3. **Comprehension**: Participants and their LAR/study partner who can provide written informed consent.
4. **Partner/caregiver**: Participants must have a study partner who has frequent interaction with the participant on a regular basis and can reliably attend study visits.

LIFE-DSR: new developments

"Parent" study: LIFE-DSR

LIFE-DSR-Goal Attainment Scaling (GAS)

LIFE-DSR-Biomarker

Thank you for your time and interest. Please reach out with questions and be on the lookout for more information!