GLOBAL MND RESEARCH ROUNDTABLE

A FIGHTMND EVENT

2025 MELBOURNE OUTCOMES DOCUMENT

TABLE OF CONTENTS

EXECUTIVE SUMMARY **02**

THE GLOBAL MND RESEARCH ROUNDTABLE JOURNEY **04**

ROUNDTABLE STRATEGY **05**
Strategy on a Page 05

GLOBAL ENGAGEMENT **06**

ACCELERATING RESEARCH IMPACT THROUGH GLOBAL COLLABORATION AND ALIGNMENT **09**
Day 1 09
Day 2 17
Next Steps 22
2025 ALS/MND Global Research Roundtable Priorities and initiatives 23

APPENDIX **24**
Appendix 1 - Global Roundtable Engagement Proposed Melbourne Sub-Initiative Grouping and Justification 25
Appendix 2 - Scope, Objectives and Givens (SOGs) 31
Appendix 3 - Scope, Objectives and Givens (SOGs) 32

EXECUTIVE SUMMARY

The 2025 Global Motor Neurone Disease (MND) Research Roundtable brought together more than 60 of the world's leading experts in MND, people with lived experience and experts from outside the MND space to accelerate research in MND. This was the second Global MND Research Roundtable event held by FightMND and it aimed to turn the seven ambitious goals identified at the inaugural event into tangible actions and initiatives.

Continuing the momentum from the 2024 Roundtable event, over the last 12 months a global engagement (>600 researchers across >50 countries) and prioritisation process identified the top five priority initiatives to focus on;

1. Create an initiative to prioritize **VALIDATION OF ALS/MND BIOMARKERS**
2. Create a globally collaborative **ASYMPTOMATIC ALS/MND INITIATIVE**
3. Create a global hub for **MORE EFFECTIVE USE OF HUMAN ALS/MND MODELS**
4. Create a strategic initiative to **INCREASE GENETIC DATA & BIOSAMPLING FROM UNDERREPRESENTED GROUPS**
5. Create a guidance for the field on **PRECLINICAL EVIDENCE NEEDED FOR EFFECTIVE CLINICAL DEVELOPMENT**

These five priorities represent a strong consensus from the field as the greatest opportunities where a strategic and co-ordinated approach would accelerate research progress and have significant impact in the field. These priorities shaped the program of discussion for the 2025 Roundtable.

To ensure robust debate and discussion participants included established and emerging key opinion leaders in the MND sector with specific research interests and expertise that aligned with the priorities. Additionally, research leaders in priority areas from outside of the MND sector were also invited to allow for learnings and opportunities to collaborate with other fields of research. Through targeted invitations, 2025 participants covered a wider breadth of the ALS/MND research community through both geography and expertise, including people with a range of lived experience (PLEx) ensuring diversity of voices and experience.

Across the two-day workshop, working both within a large group and smaller sub-groups, participants mapped the landscape and challenges and identified incentives and barriers within priority areas before developing approximately 5-9 feasible actions and activities. The actions were elaborated in more detail, defining desired outcomes, key tasks, timing, stakeholders, resources and infrastructure needed, and interdependencies with other actions or priorities. Each group then presented their prioritised action(s) in a short five-minute pitch.

The key outcomes of the Roundtable were the development of five initiatives to address the priorities of the MND research field.

- Biomarkers - Establish the **VIBRANT** Working Group - **V**irtual **I**nternational **B**iorepository **A**lliance **N**e**T**work consisting of biomarker & biorepository leaders
- Establish the **AWARE** Working Group – **A**LS **W**orldwide **A**symptomatic **R**esearch **E**ndeavour consisting of willing leaders of existing asymptomatic research cohorts
- Establish the **iGLOBAL**S Working Group consisting of ALS-relevant primary human cell model experts
- Establish the Global **SEED** Working Group – Global **S**outh **E**ngagement to **E**nhance **D**iversity in ALS/MND consisting of leaders from Global South countries with research or near research capacity
- Establish the **TAP** Working Group – **T**herapeutic **A**ccelerator Panel consisting of diverse experts with broad representation from across consortia, industry, regulatory, academic and patient communities.

Initial thoughts at the start of day two of the workshop were that tackling the five priorities would be too ambitious and the field should choose to focus on 1-2 priorities rather than driving all five forward. The work completed on day two of the workshop led to the development of tangible actions and activities under each initiative. These were mapped out and included both short (quick wins) and longer-term outcomes and through feedback and synthesis conversations there was strong consensus from participants that all five initiatives represent exciting opportunities for the MND field to work collaboratively and strategically to accelerate research discovery and translation, and to improve outcomes for those living with MND.

Additional outcomes from the Roundtable included robust debate and discussion on the importance and necessity of global collaboration for global impact. While there is still the need for further debate, the consensus from the group was that while some actions

within the initiatives require a globally collaborative effort (in particular AWARE and VIBRANT) some activities can be delivered on a local and/or regional level and outcomes expanded globally. There was very strong consensus on the desire to collaborate and connect to develop new networks and supercharge resources for research in under-represented countries.

Lastly, there was good discussion on how to progress each initiative including the establishment of working groups, resourcing and an emphasis on action, not words. Across both the 2024 and 2025 Roundtable participants and the MND field more broadly, there is commitment to work more effectively and efficiently to improve outcomes for people with MND.

The key next steps are to drive the initiatives forward, including communication and engagement/ awareness of outcomes with the broader MND field, the establishment of working groups to shape next steps and road map and and on-going commitment from FightMND, ALS Canada and any welcomed collaborators, to continue to drive strategic action in the fight against MND.

THE GLOBAL MND RESEARCH ROUNDTABLE JOURNEY SEPT 2024–AUGUST 2025

The original Global MND Research Roundtable was held in September 2024 to identify opportunities *"to accelerate discoveries and find effective treatments and a cure for MND through global alignment and international collaboration"*.

The top four global barriers to MND research translation were identified prior to the event through surveying the attendees;

- Disease heterogeneity - a barrier to effective diagnosis, treatment and understanding of the disease across different populations.
- Biomarkers and diagnostic markers - need improvement for better diagnostics, treatment and research outcomes.
- Understanding disease fundamentals - a key priority for developing effective treatments.
- Patient stratification and classification - important for achieving more precise and effective research and treatment approaches.

Discussions focussed on these four areas over two days and produced seven ambitious goals to take forward;

1. Global centralization of BIG DATA
2. Global collaboration of BIOBANKING
3. Global approaches to STUDYING ASYMPTOMATIC DISEASE
4. Best practice recommendations for the use of PRECLINICAL ALS/MND MODELS
5. A standardized approach to translation through a DECENTRALISED HUMAN ALS/MND MODEL CORE
6. Best practice recommendations for clinical and preclinical BIOMARKER RESEARCH
7. An ALS/MND MASTER CLINICAL TRIAL PROTOCOL

Following the 2024 Roundtable, Drs Bec Sheean (FightMND), David Taylor (ALS Canada) and Gethin Thomas (at that time MND Australia but now with FightMND) were tasked with driving the next steps to progress the Roundtable work.

ROUNDTABLE STRATEGY

A "strategy on a page" was then developed and presented at an International ALS/MND Symposium satellite meeting in Montreal in December 2024. Attending this meeting were Roundtable participants as well as additional Key Opinion Leaders (KOLs) not able to attend the initial Roundtable, representatives of the International Alliance and other ALS/MND organisations around the world.

The Montreal meeting also emphasised the following points:

- the strategy is good, but too ambitious
- a reiteration of the concerns over stifling innovation by too much standardization and harmonization
- need for a global initiative to engage more widely across the ALS/MND research global community

STRATEGY ON A PAGE

OUR VISION is a world without ALS/MND

OUR MISSION is to accelerate research through global collaboration and alignment

OUR PRINCIPLES guide our approach to everything we do:

We are **COURAGEOUS**

We are **GLOBALLY MINDED**

We value **MEANINGFUL PARTNERSHIPS**

We will **BALANCE COLLECTIVE AND INNOVATIVE THOUGHT**

We are **INCLUSIVE & RESPECTFUL**

We work with **URGENCY**

We work **EFFICIENTLY**

OUR STRATEGIC FOCUS AREAS are harmonisation and standardisation, and OUR 7 AMBITIOUS GOALS are

1. Global centralization of **BIG DATA**
2. Global collaboration of **BIOBANKING**
3. Global approaches to **STUDYING ASYMPTOMATIC DISEASE**
4. Best practice recommendations for the use of **PRECLINICAL ALS/MND MODELS**
5. A standardized approach to translation through a **DECENTRALISED HUMAN MND/ALS MODEL CORE**
6. Best practice recommendations for clinical and preclinical **BIOMARKER RESEARCH**
7. An **ALS/MND MASTER CLINICAL TRIAL PROTOCOL**

OUR KEY STRATEGIC ENABLERS will help us to get the job done:

- An engaged **COMMUNITY** of people affected by MND/ALS at every level of governance
- A **COMPLEMENTARY** approach to the current landscape to avoid duplication of efforts
- **COLLABORATIVE** resourcing of funding and in-kind contributions

GLOBAL ENGAGEMENT

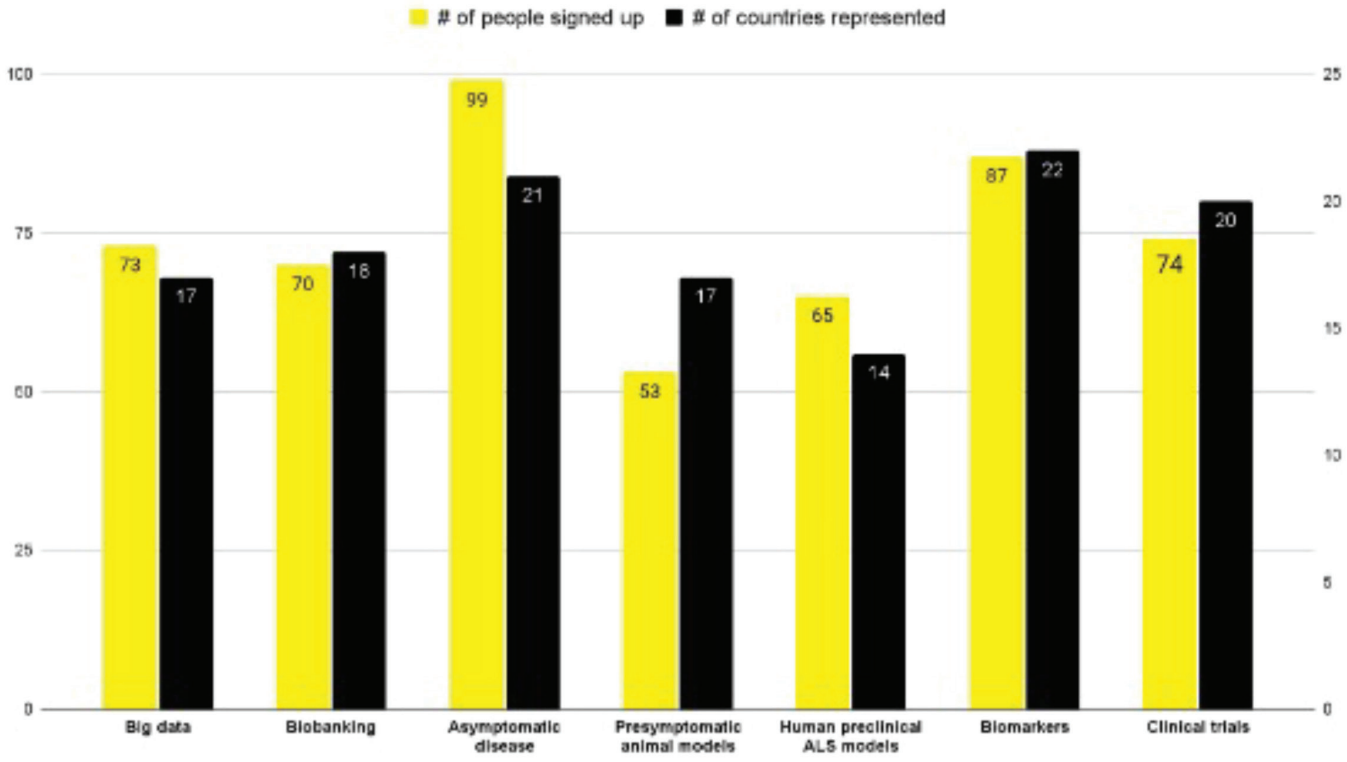
Based on feedback in Montreal, a decision was made to engage a wider group of ALS/MND researchers and clinicians, and to focus on more achievable outcomes that fit within the strategy designed at the 2024 Roundtable.

This engagement took the form of a series of video calls in two stages. The first stage was to host a video call centred on each of the seven goals where specific actions outlined in Melbourne, in addition to newly solicited ideas, were anonymously voted on to prioritize work that was widely felt to be of globally impactful significance.

The second engagement stage consisted of four additional video calls with voting on the top 18 ideas from across the seven initial calls. These additional calls were specifically targeted to broaden the reach across the wider ALS/MND research community through time zone scheduling and distribution through contacts in under-represented regions, in addition to the International Alliance, PACTALS and ELATAM.

In total we reached out to ~600 researchers across more than 50 countries and we had over 150 different researchers participate in the calls. From these results, we created five proposed priority areas which formed the basis for the work for the 2025 Roundtable. The detailed basis for justification of these priorities can be found in Appendix 1 titled *"Global Roundtable Engagement Proposed Melbourne Sub-Initiative Grouping and Justification"*.

The graph below represents the engagement across the seven goals in the global call process.



THE PRIORITY AREAS

1. Create an initiative to prioritize **VALIDATION OF ALS/MND BIOMARKERS**
    a. Including a strategy for timely incorporation into ***better subgrouping for clinical trials***
    b. Potential consideration of a ***focus on getting people into trials earlier***
2. Create a globally collaborative **ASYMPTOMATIC ALS/MND INITIATIVE**
    a. Start with a high-level ***registry of variants being followed***
    b. Work towards development of a ***minimum common dataset***
    c. Gauge evolving ability to connect existing initiatives and datasets as collaboration grows
    d. Potential consideration of ***novel approaches to finding initiating factors for "sporadic ALS/MND, studies of unique familial cases (e.g. twin studies, phenocopy studies, etc.) and work to identify genetic modifiers of ALS/MND***
        i Working Group contributors for consideration would include individuals who put their name forward for **ASYMPTOMATIC ALS/MND**
3. Create a global hub for **MORE EFFECTIVE USE OF HUMAN ALS/MND MODELS**
    a. Start with developing a ***central resource of publicly available, validated IPS lines***
    b. Consider building an ***academic or CRO-based independent validation network***
    c. Potential consideration of ***creating best practice recommendations for the creation of human ALS/MND models***
4. Create a strategic initiative to **INCREASE GENETIC DATA & BIOSAMPLING FROM UNDER-REPRESENTED GROUPS**
    a. Including a ***best practices mechanism*** to ensure interoperability of all new collection
    b. Informed by a ***globally collaborative data dictionary for collection in ALS/MND*** that includes consideration of ***individual types of data (-omics, imaging, wearables, etc.)***
    c. Potential consideration of **development of a standard template agreement for biosample sharing and organization of a global summit to discuss big data and biorepositories**
5. Create a guidance for the field on **PRECLINICAL EVIDENCE NEEDED FOR EFFECTIVE CLINICAL DEVELOPMENT**
    a. Potential consideration of ***best practice recommendations for the creation of human ALS/MND models, developing guidance or harmonized key metrics on reporting of trial results, emphasizing target engagement and biomarkers for translation in pre-clinical research and organization of global versions of successful NEALS/TRICALS/PACTALS initiatives***

These priorities were then further tested prior to the 2025 Roundtable through a survey. The survey was sent to all attendees and those who had participated in the global engagement calls.

SURVEY OUTCOMES

To confirm whether the 2025 Roundtable priorities resonated with the research community, we asked whether respondents were satisfied with the justification of the five priority areas and accompanying actions to serve as a starting point for work in Melbourne.

One response suggested more focus was needed on palliative care and socioeconomic aspects of ALS/MND care. Although we recognise these are critically important areas, they are considered out of scope for the Roundtable. A second response questioned whether all five priorities should be considered equally important. This concern was earmarked to be addressed early in the program. All other respondents agreed with the prioritization and justification of the priority areas.

The following survey questions then asked respondents to rank each of the five priorities in terms of potenital impact, feasibility and their desire to work on that priority.

Rankings are averages (1 = highest priority, 5 = lowest priority)

Melbourne = participants at the Rroundtable (n=43). Combined = Melbourne + external input (remote voters)(n=77).

MELBOURNE	AVG RANK	COMBINED	AVG RANK
IMPACT			
Biomarkers	1.7	Biomarkers	1.7
Human Models	3	Human Models	3
Asymptomatic	3.3	Asymptomatic	3.3
Underrepresented	3.4	Underrepresented	3.5
Preclin. Evidence	3.7	Preclin. Evidence	3.6
FEASIBILITY			
Biomarkers	2.7	Biomarkers	2.6
Asymptomatic	2.9	Asymptomatic	2.9
Underrepresented	3.1	Underrepresented	3.1
Preclin. Evidence	3.1	Preclin. Evidence	3.1
Human Models	3.3	Human Models	3.3
INTEREST			
Biomarkers	2	Biomarkers	2
Asymptomatic	3.1	Asymptomatic	2.9
Human Models	3.1	Human Models	3.2
Underrepresented	3.3	Underrepresented	3.4
Preclin. Evidence	3.4	Preclin. Evidence	3.6

Results were consistent across both Melbourne attendees and other global call participants.

Using average rankings from a scale of 1 (highest) to 5 (lowest), the biomarker priority area was clearly considered the most impactful with the other four initiatives considered to have lower, but similar impact to each other. Work on human models was a clear second in terms of impact. The biomarker priority area was considered slightly more feasible than the other initiatives, with the asymptomatic disease priority as a clear second.

For the Melbourne Roundtable, participants were assigned to working groups for the priorities they ranked either 1 or 2 in their interest to work on.

Overall, pre-meeting survey results demonstrated that the five priority area rankings, in terms of impact, feasibility and interest, largely mimicked the scoring from the eleven global engagement calls, and may represent a means of later prioritisation.

2025 ROUNDTABLE PARTICIPANTS

A deliberate effort was made to diversify the participants for 2025 to cover a wider breadth of the ALS/MND research community through both geography and expertise. We welcomed Australian and international discovery and clinical researchers, neurologists, clinical trial experts, funding organisations, pharmaceutical companies, ALS/MND Associations as well as experts from other research areas with experience in global collaboration. A number of representatives from PACTALS attended as well as the Board Chair, CEO, and Research Director of the International Alliance of ALS/MND Associations. Critically, people with a range of lived experience (PLEx) also attended. Approximately 50% of the 60+ attendees did not participate in the 2024 Roundtable.

PROGRAM DEVELOPMENT AND DELIVERY

The event was jointly facilitated by Amanda Nolan, Principle of Nolan Consulting, and James van Smeerdijk, Principle of Atticus Now, supported by the Atticus Now team.

The program was based on the Scan-Focus-Act model; a three-part approach to gathering information on the background and key issues, using that information to decide what's worth exploring more rigorously, and testing whether the areas or ideas of focus can lead to useful results.

The Scope, Objectives and Givens (SOGs) form the backbone of the program. The SOGs were co-designed and developed with David Taylor, Gethin Thomas and Bec Sheean. These can be found in Appendix 2 *"Scope, Objectives and Givens (SOGs)"*.

ACCELERATING RESEARCH IMPACT THROUGH GLOBAL COLLABORATION AND ALIGNMENT

DAY 1

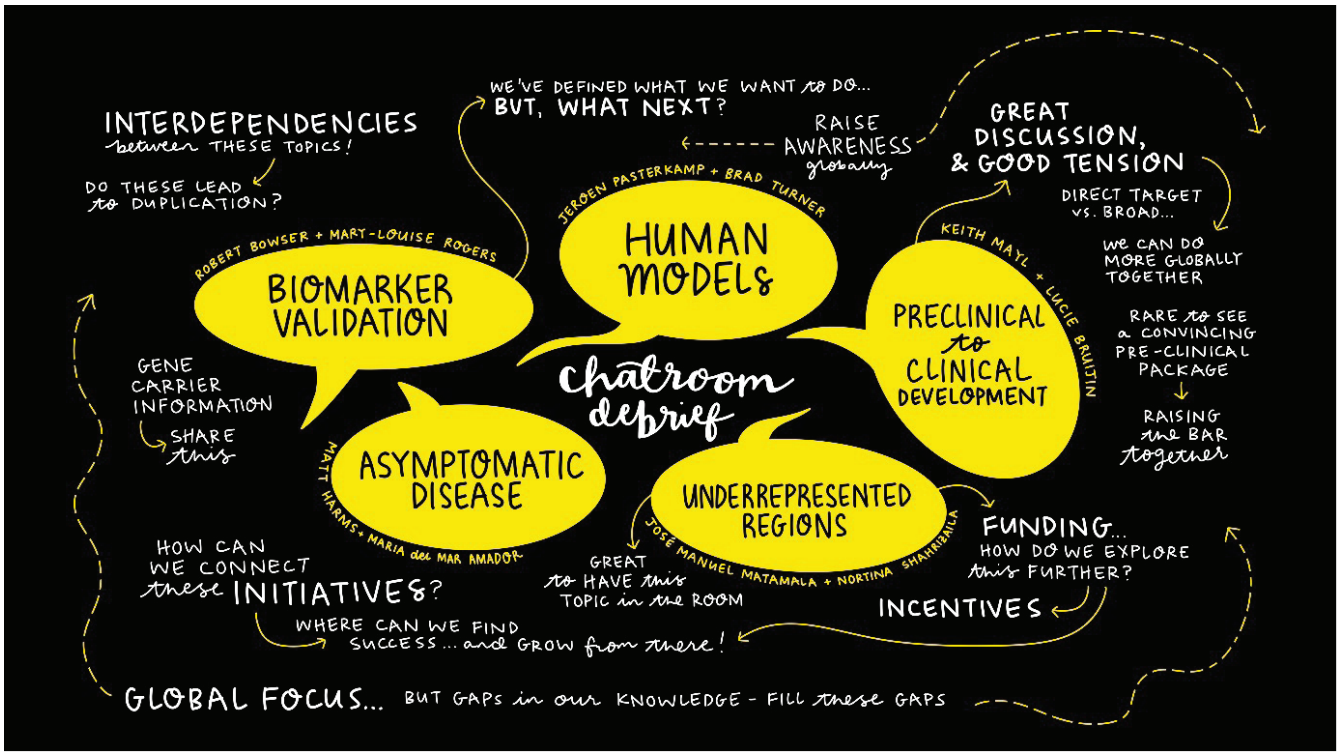
Following the initial Roundtable which discussed "The global barriers to research translation", the desired outcome for the 2025 Roundtable was to develop specific initiatives that could drive impact through overcoming these barriers.

With 50% of the delegates new to the Roundtable, a Knowledge Wall was presented which outlined the Roundtable work to date, covering the outcomes of the 2024 Roundtable, development of the strategy, the meeting in Montreal and the outcomes of the global engagement process.

The first stage of the program was to introduce the priority areas through summarising the current landscape in that area, identify the most critical and solvable challenges for the next 24 months, and map existing work, gaps, and success measures.

To help drive discussion, each priority area was represented by two "Champions", who were respected Key Opinion Leaders (KOLs) in that area.

- Biomarker validation – Robert Bowser, PhD (Barrow Neurological Institute, USA), Mary-Louise Rogers, PhD (Flinders University, Australia)
- Asymptomatic disease – Maria del Mar Amador, MD (Paris Brain Institute, France), Matt Harms, MD (Columbia University, USA)
- Human models – Jeroen Pasterkamp, PhD (UMC Utrecht, Netherlands), Brad Turner, PhD (Florey Institute, Australia)
- Underrepresented regions – José Manuel Matamala, MD (University of Chile, Chile), Nortina Shahrizaila, MD (University of Malaysia, Malaysia)
- Preclinical to clinical development – Lucie Bruijn, PhD, MBA (Novartis, UK), Keith Mayl, MD PhD (Argenx, UK) Work



TEAM LISTS				
1. BIOMARKER VALIDATION	**2. ASYMPTOMATIC DISEASE**	**3. HUMAN MODELS**	**4. UNDER-REPRESENTED REGIONS**	**5. PRECLINICAL TOCLINICAL DEVELOPMENT**
Allan McRae	Bernd Merkel	Ariel Forrai	Adriano Chio	Anthony Akkari
Andrea Malaspina	Calaneet Balas	Brad Turner Ammar	Al-Chalabi	Cathy Blizzard
Angela Genge	David Taylor	Jeremy Shefner	Cathy Cummings	Dongsheng Fan
Davor Stanic	Emma Scotter	Jeroen Pasterkamp	Eleanor Ramsey	Jennifer Hollands
Gerald Pfeffer	Jane Milne	John Clark	Ginny Sargent	Julie Atkin
Leonard van den Berg	Julia Morahan	Lezanne Ooi	José Manuel Matamala	Keith Mayl
Mary-Louise Rogers	Maria del Mar Amador	Ludo Van Den Bosch	Min-Yin Yap	Lucie Bruijn
Matthew Kiernan	Matt Harms	Lyle Ostrow	Atcharayam Nalini	Manish Raisinghani
Matthew Webb	Michelle Kouspou	Martina de Majo	Natalie Gauld	Nick Cole
Robert Bowser	Paige Higgins	Michael Dobbie	Nortina Shahrizaila	Paul Wright
Ruben van Eijk	Sheila Agustini	Phil Camden	Paul Talman	Rebecca San Gil
Seung Kim	Thanuja Dharmadasa	Shyuan Ngo	Paula Trefiak	Sarah Bennett
	Wern Su		Rochelle Tobin	Tina Soulis

WORK ROUND #1: CHALLENGES, GAPS, SUCCESS

Delegates then moved into the first work round for the Roundtable and were asked to identify “What are the most critical, solvable challenges in this priority area over the next 24 months?

This was done through answering a series of questions;

1. What are the most critical, solvable challenges in this Priority area over the next 24-months?
2. What relevant work is already being done on the challenges? What are the key gaps?
3. What would success look like for each of these challenges? How would we measure it?

These were answered individually, then as a group, then clustered into themes. A scout round then enabled delegates to move around the room and provide feedback on the outcomes for different priorities and identify new ideas, overlaps and interdependencies.

Following this round the priority groups reformed and discussed feedback. A voting round was then held based on Impact, Feasibility, Leverage (i.e. does it build on existing work?), Collaboration Potential and Momentum (is there energy to pursue this?).

The voting served to highlight points with strong alignment as well as contention. Collated outcomes following group synthesis are below;

BIOMARKERS

CHALLENGES

- No universal approach
- No agreed validation process
- Limited sharing of resources
- Low quality validation/development studies
- Not enough $$
- Too much heterogeneity in samples
- How to collect for future needs
- Differing levels of knowledge and technology
- Need paired blood/brain samples
- Set success bar too high, too early for prospective biomarkers

GAPS

- No standardised SoPs
- Which platforms to use for assays?
- Accessing industry samples
- Lack of wide representation in samples
- What does replication look like?

SUCCESS

- Subgroup-specific diagnostics
- Exploratory biomarkers in trials
- Industry samples widely available
- More validated biomarkers
- Standardised NFL measures
- Surrogate biomarkers
- New therapies predicted by biomarkers
- Central biosample repository
- $$ to support sharing

ASYMPTOMATIC DISEASE

CHALLENGES

- Harmonisation/how to combine smaller studies
- How to collaborate better
- Awareness in affected families
- Interest/priorities – motivation/people at risk
- Fractured goals
- $$$
- Need a global database
- Siloed biobanks
- Availability of genetic counsellors

GAPS

- Not enough data/study participants (underpowered)
- Not enough collaboration

SUCCESS

- Large cohorts/suitably powered results
- Earlier intervention/prevention
- Higher risk individuals make informed decisions
- Identify upstream targets
- Better biomarkers and understanding of mechanisms/process of disease

HUMAN MODELS

CHALLENGES

- Heterogeneity/reproducibility
- Need to improve consensus on what is best
- Standardised protocols/reagents
- Need defined IPS QC
- Accuracy in human disease modelling/don't know what cells in human disease are doing
- Long timeline to mature cultures/labour intensive/need high level of expertise

GAPS

- No clear idea of which models are best
- What iPSC resources are out there?
- Collating existing literature/knowledge
- How to report negative results
- No single model sufficient

SUCCESS

- Capture disease heterogeneity/human biology
- Cells from all global regions
- Standardised protocols
- Standardised pool of iPSC lines
- PLEx engagement/understanding
- In vitro clinical trials
- Meta-analysis
- White paper guideline
- Standardised set of lines
- Network of resources
- Specific meetings/training/conferences
- Engage funders/journal to incentivise the work/compliance

UNDER-REPRESENTED REGIONS

CHALLENGES

- Establishing a network
- Meeting regions where they are at
- PLEx lead
- Begin with a single centre
- Lack of standardisation
- Resources - $$, human, unequal across countries and within countries
- Need to diagnose patients first
- No biorepositories
- Research not priority over other care pressures
- Lack of engagement – treatments will be global

EXISTING WORK

- Secretariat support – ELATAM, ALSAfrica-Net
- Defined national leaders
- Discovery meetings (virtual)
- International Alliance brings together networks

GAPS

- No registries
- No representation for all countries
- Not represented at top table
- Lack of $$
- Lack of industry/infrastructure/trial readiness at potential sites
- Poor consideration of cultural factors

SUCCESS

- Global data dictionary
- Infrastructure for clinical trials
- Globally implemented genetic counselling/ guidelines
- Global mentorship program/fellowships
- More registries – local/national/regional
- Trial ready sites/Inclusion in clinical trials (2 sites/region)
- Equality of global network care/treatment
- New discoveries

PRECLINICAL EVIDENCE FOR EFFECTIVE TRANSLATION

CHALLENGES

Potentially Solvable

- Need target engagement biomarkers
- Better opportunities to publish negative data
- Proven/established models for defined roles e.g. PK/PD or biology
- How to use AI

- Lack of collaboration/openness
- Go/no-go decision to progress to clinic – not always science – sometimes $$/VC-driven
- Need better academic-industry collaborations
- Slow and expensive to progress preclinical studies to clinical trial
- Inappropriate use of animal models
- Survival as endpoint in animal models inappropriate
- Need global consensus

Not Solvable

- ALS as a rare disease = small drug market for pharma = less attractive
- Fear of failure – consequences if drug does not progress
- Frustration from previous failures
- Mouse vs human differences
- Need to challenge current trends/dogma

GAPS

- Databanks/repositories/registries – need linking – AI option?
- Need better biomarkers
- What models are missing?
- What makes a good model? Consensus on which models to use/for what/human-animal correlation
- Need animal models to go beyond biology – e.g. PK etc
- Access to technical/translational expertise – advice – what works/doesn't

SUCCESS

- Publishing negative data
- Global dataset
- Better data sharing
- Better access for patients to treatment/more treatments/repurposed drugs
- Better translation rates
- Use of new technology
- More accurate disease modelling
- Reproducibility
- More interest – fatter pipeline

DEBATE: GLOBAL COLLABORATION AND IMPACT

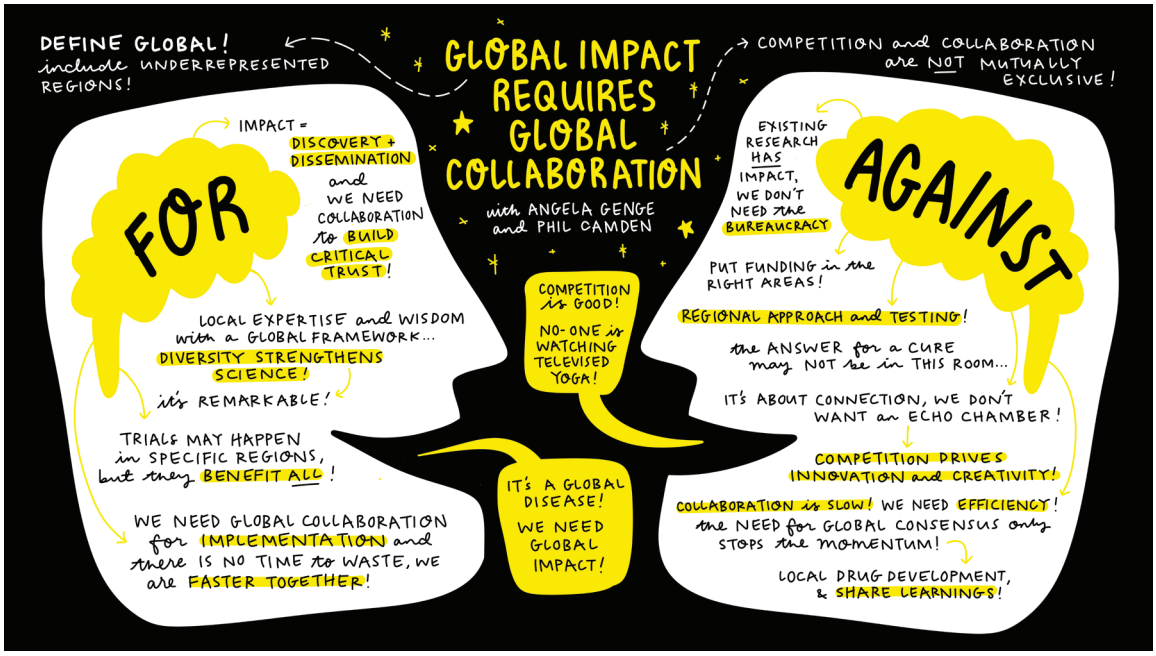
Following the initial scan of the problems, a debate was held to explore the nature of global collaboration with the motion *"Global impact requires global collaboration"*. The objective was to explore the idea that global outcomes do not always need full global involvement but rather the potential impact must be global and not confined to certain groups.

Two teams participated in the debate, and each provided thoughtful commentary to consider as the Roundtable Initiative moves forward.

Team For - Ammar Al-Chalabi, Julia Morahan, Lyle Ostrow

Team Against - Cathy Blizzard, Nortina Shahrizaila, Ruben van Eijk

Key take aways from the debate were that some global problems can be solved locally and implemented globally, while others require a global collaborative approach.



SCISSOR PAPER ROCK-OFF

To energise everyone after some intense sessions diving deep into the challenges for each priority, we held a Scissor Paper Rock-off with Professor Paul Talman edging FightMND's Ariel Forrai in a hard-fought final much to their respective joy and misery.

INDIVIDUAL PERSPECTIVES: STAKEHOLDER MAPPING

The final session of the first day was an Individual Perspectives Exercise where attendees were tasked to reflect on their own personal barriers, incentives and potential trade-offs to engaging in global collaboration for MND research. These reflections then formed the basis for a stakeholder mapping exercise for each of the priorities. This included scanning the key stakeholders relevant to their priority area, brainstorming their barriers and incentives to engage, and identifying where gaps in knowledge remain.

INCENTIVES

- What would global collaboration help you achieve that's hard to do alone?
- What return would your organisation expect for the time and resources invested?

BARRIERS

- What makes collaboration harder than it should be – in your context?
- What have you seen go wrong in previous collaborative efforts?

TRADE-OFFS

- What costs or compromises would your organisation struggle to accept?
- What kinds of decisions feel hardest to make together across borders?

The individual perspectives were then collated within each group to identify stakeholders not in the room and common barriers, incentives and gaps.

An additional discussion was also held with attendees grouped via their specialisation; clinicians, lived experience, industry, ALS/MND organisations, non-ALS/MND organisations and discovery researchers. Similar outcomes were identified from this discussion as in the priority groups and were incorporated into the summary below.

BIOMARKERS

STAKEHOLDERS

- **PHARMA/INDUSTRY/VC INVESTORS**
  - **Barriers**
    - IP/ownership
    - Funding
    - Small disease/population
  - **Incentives**
    - Recognition
    - De-risk development costs
    - De-risk investments
  - **Gaps**
    - Validation/reproducibility of current biomarkers
    - Compelling story that will appeal to funders/government
- **REGULATORS**
  - **Barriers**
    - Too rigid/strong authority
  - **Incentives**
    - Good story
    - Reduce healthcare costs
  - **Gaps**
    - Compelling story that will appeal to funders/government
- **AI/BIG DATA ANALYTICS GROUPS**
  - **Barriers**
    - Harmonisation of data
    - Lack of truly large datasets
  - **Incentives**
    - Global Impact
    - Control/access to data
  - Gaps
    - Need more data
    - Who brings data together?
    - No wide standardisation
- **PALS/PLEX**
  - **Barriers**
    - Burden – short-time
  - **Incentives**
    - Impact on drug development
    - Knowledge of disease
    - Altruism/contribution to science
  - **Gaps**
    - Access for poorer countries

ASYMPTOMATIC

STAKEHOLDERS

- **PEOPLE WHO HAVE NOT BEEN TESTED/ IDENTIFIED**
  - **Barriers**
    - Finding gene carriers/asymptomatic patients
    - Genetic counselling/testing access
    - Family dynamics
    - Geography
    - Cultural differences
  - **Incentives**
    - Access to treatments
    - Life planning
    - Family planning
    - Family risk
    - Altruism
  - **Gaps**
    - Global information on testing (why/ how/who/best practice)
    - Where are they/How many?
    - Risk factors
- **RESEARCHERS UNDERTAKING THEIR OWN ASYMPTOMATIC DISEASE RESEARCH**
  - **Barriers**
    - Competing opinions/process
    - Compatibility of data/ethics/ regulatory requirements
    - Institutional incentives
    - Protecting participants
    - Collaboration/communicating with industry
  - **Incentives**
    - Larger “n”
    - Publications
    - Altruism
    - Representation of other groups
  - **Gaps**
    - Who is doing these studies?
- **Under-represented countries**
  - **Barriers**
    - Funding
    - Infrastructure (basic care levels)
    - Communication
    - Cultural differences
    - Trauma
  - **Incentives**
    - Funding
    - Seat at the table
    - Depends on culture
  - **Gaps**
    - Which countries are not represented currently
- **GENETIC COUNSELLORS**
  - **Barriers**
    - Lack of counsellors/time/ALS/MND expertise
  - **Incentives**
    - Share expertise
    - Altruism/personal motivation
    - Recognition
    - Publications
- **POLICYMAKERS/GOVERNMENT**
  - **Barriers**
    - Competing priorities/urgencies
    - Constituent priorities/urgencies
    - Awareness/understanding

- **Incentives**
  - Votes
  - Altruism
- **Gaps**
  - Is MND a priority for government
  - How to raise MND profile with people at risk?

HUMAN MODELS

STAKEHOLDERS

- **INSTITUTES**
  - **Barriers**
    - Administrative barriers (MTAs etc)
  - **Incentives**
    - Funding
    - Reputation
    - Recognition
    - Community impact
    - Sustainable infrastructure
- **UNDER-REPRESENTED PATIENTS/PLEX**
  - **Barriers**
    - Cultural (sharing tissues/cells)
  - **Incentives**
    - Education
    - Inclusion
    - Communication of results
    - Outreach
    - Recognition
    - Self-determination i.e. choose to donate cells etc
  - **Gaps**
    - Rate of disease progression
    - Specific cultural beliefs
- **REGULATORY BODIES/GOVERNMENT**
  - **Barriers**
    - Different rules/regulations
    - Rare disease – lower priority with low patient numbers
  - **Incentives**
    - Reduced care costs
    - Socioeconomic benefit
    - Increased lobbying power
- **CONTRACT RESEARCH ORGANISATIONS**
  - **Barriers**
    - Revenue model
    - IP ownership
  - **Incentives**
    - $$
- **GENERAL PUBLIC**
  - Barriers
    - Public opinion – animal models/ stem cells
  - **Incentives**
    - Education/outreach/information
    - Reduce cost burden of disease
  - **Gaps**
    - Knowledge of MND
    - Opinion/understanding of iPSCs

UNDER-REPRESENTED REGIONS

STAKEHOLDERS

- Government/funders
- Regulators
- International organisations e.g. WHO
- Regions beyond PACTALS and ELATAM e.g. Africa, Middle East, Russia
- NFPs/charities
- Health services
- PLEx from underrepresented regions

BARRIERS

- Not a funding priority
- Not on regulators'' radar
- Under-resourced
- Legal barriers
- Bureaucracy
- No travel grants for PLEx
- Philanthropic organisations having no connection to ALS/MND
- Under-represented groups excluded from international collaborations due to their government not part of consortium

INCENTIVES

- Opportunity to participate in clinical trials
- Recognition and profile
- Transferrable learning
- Improved care
- Improved economic impact

GAPS

- Unknown connections within government/ funders/regulators
- Inadequate funding
- Publicity
- List of potential donors/funding bodies
- Not knowing the hook

PRECLINICAL EVIDENCE FOR CLINICAL TRANSLATION

STAKEHOLDERS

- Regulators/ethics boards
- KOLs/researchers
- Funders/investors/VC/pharma
- PLEx
- Academic partners/institutions
- CROs
- NGOs/NFPs

BARRIERS

- Diverse regulatory frameworks
- Large geographical footprint
- Lack of agility
- Non-compelling data

- Lack of disease understanding
- Unrealistic expectations
- High failure rate/Valley of Death
- Limited commercial opportunity
- Prioritisation vs other diseases

INCENTIVES

- Stronger evidence base/publications
- Consensus across ALS/MND community
- Patient-centric drug development
- Compelling data
- Engagement/Invites to conferences
- Access to resources to accelerate timelines/ review processes
- Share impact of preclinical data
- Early consultation

GAPS

- Unclear regulatory expectations/requirements
- Engagement forums
- Endpoint development

Across the priorities, a number of common themes could be seen;

For Barriers;

- Raising the profile of MND to make it a priority for government/regulators/funders
- Constantly shifting and/or unclear regulatory requirements with increasing administrative burden
- Funding resources
- Lack of global standardisation/consensus across many areas
- Competing opinions/priorities

For Incentives;

- Improved care and treatment options
- Consensus across ALS/MND community
- Better outcomes for PLEx
- More resources/funding
- Altruism
- Recognition

For Gaps;

- How to best/better engage with government/ regulators
- How to bring all data together
- Representation/involvement of under-represented regions

The barriers, incentives and gaps outlined for each priority area will be thoughtfully considered when moving work forward in each of the respective areas

ONE IDEA

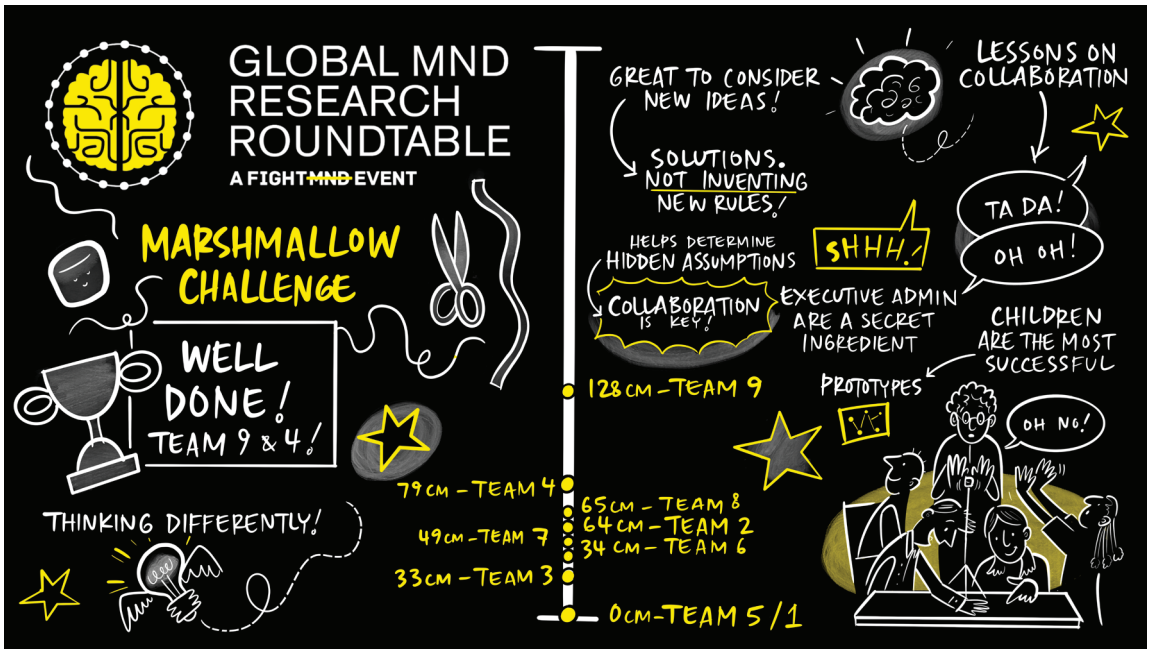
To end Day One, delegates were asked to reflect on the day's events and write down the one idea that inspired or stood out to them most. The most common themes were;

- Harmonisation/standardisation
- Focus on impact
- Global collaboration
- Global consensus
- Sharing, trust
- Engaging with regulators

DAY 2

MARSHMALLOW CHALLENGE

Day 2 began with the Marshmallow Challenge, a hands-on activity where teams were instructed to build the tallest freestanding structure topped with a marshmallow using simple materials. In the debrief, themes of collaboration, rapid prototyping, and testing assumptions emerged. Success required constant communication, early experimentation rather than over-planning, and a willingness to learn quickly from failure – all relevant lessons in the context of global MND research collaboration.



DAY 1 REFLECTIONS

Reflecting on Day 1, a number of points were raised;

- There was great energy in the room and a will to get things done
- The five priorities have a number of interdependencies
- Do not be hesitant with coming forward with challenging ideas – we need to make sure potential issues are raised early carefully considered
- We need actionable outcomes
- Think globally

WORK ROUND #2: DEVELOPING THE SOLUTIONS

Having completed the “Scan” and “Focus” stages we then progressed to “Act” with the next task being to start “Developing the Solutions”

To instil belief that meaningful outcomes would come from the Roundtable, a commitment was made by FightMND and ALS Canada to jointly support a 1.0 FTE to support the priorities developed.

Within each priority group, individuals identified “What are the key actions you would like to take within a two-year horizon?” These were then refined as a group into 5-9 actions. The actions were elaborated in more detail, defining desired outcome, key tasks, timing, key stakeholders, resources and infrastructure needed, and interdependencies with other actions or priorities.

Following a scout round to receive feedback from the other groups in the room, each group then presented their prioritised action(s) in a short five-minute pitch and Q&A session to justify support from the previously announced FTE.

BIOMARKERS

ACTIONS:

- **BEAST – Biomarker Education and Scientific Training**

  Key Tasks;
  - International Scientific Exchange program
  - Selection Committee
  - SoPs/training for best practice

- **Expanding iGLOBALS (see Human Models)**

  Key Tasks;
  - Biological samples and clinical data for iPSCs from under-represented regions
  - Establish guidelines for sample collection with minimum effort to biobank for future iPSC generation

- **Outcomes ALS - Committees to prioritise biomarkers for validation testing**

  Key Tasks;
  - Create committee and members (several months)
  - Prioritise biomarkers for replication/ validation studies)
  - NfL measures across platforms using large sample set that includes asymptomatic carriers

- **VIBRANT – Virtual International Biorepository Alliance Network**

  Key Tasks;
  - Identify global biorepositories
  - Define types of data and biosamples collected
  - Create searchable database that end users can identify location of samples of interest
  - SoPs for biorepository efforts

PITCH:

- **Why**
  - Need more effective biomarkers to find more effective treatments
- **How**
  - VIBRANT
    - Define SoPs for biorepositories to be used globally
    - Bring in major stakeholders to harmonise
  - Outcomes ALS
    - Prioritise biomarkers for replication/ validation
    - Studies across global community
    - Use samples from VIBRANT
  - RFA for Validation Studies
    - NfL as proof of concept
- **Success**
  - Define which people to treat
  - Accelerated trials
  - Improved treatments for ALS/MND
- **Why Us/Why Now?**
  - We already have biorepositories around the globe and large numbers of candidate biomarkers

ASYMPTOMATIC

ACTIONS:

- **Genetic counselling/genetic testing guideline white paper**

  Key Tasks;
  - Education/explanation around current genetic testing practices
  - Review and standardisation of genetic testing
  - White paper guidelines

- **Navigator**

  Key Tasks;
  - Steer interested gene carriers to studies/ support groups/clinicians
  - Provide education around genetic counselling and clinical care

- **Registry of Registries**

  Key Tasks;
  - Map landscape
  - Connect teams
  - Aggregate participant/sample numbers, methods, expertise
  - Coordinate registries

- **Study Toolkit**

  Key Tasks;
  - Survey methods from existing studies
  - Develop toolkit
  - Maintain up-to-date

PITCH:

- **Why**
  - To cure, manage and prevent MND by looking in places we haven't examined effectively i.e. disease commencement/ prodromal stage
  - Many diseases have benefited from such an approach
  - Earliest stage in disease ideal to educate and intervene
- **How**
  - AWARE - Asymptomatic Worldwide ALS Research Endeavor
  - Coalition of collaborators
    - RoR – Registry of Registries
      - Landscape map
      - Who is not represented
    - Study Toolkit
      - Harmonisation
      - Common minimal dataset
      - Community consultation
      - Templates

- Mega-Analysis
  - Biomarkers
  - Prediction models
  - Risk factors
- Navigator will direct participants into RoR and Mega-Analysis

- **Success**
  - Agency over ALS from the earliest phase
    - For carriers
    - For researchers/clinicians
    - For industry
- **Why Us/Why Now?**
  - Silos are proliferating
  - Early enough to impact implementation to enable cross-comparison
  - Therapies are coming/already here
  - Single biggest opportunity in ALS/MND to prevent disease i.e. A World Without ALS/MND

HUMAN MODELS

ACTIONS

- **iGLOBALS – The Global iPSC Challenge**

  Key Tasks;
  - Curate panel of ~ 10 iPSC lines
  - Advertise challenge and fundraise
  - Centralised distribution of reagents
  - Select sites (both existing initiatives and under-represented regions)
  - Protocol from METALS (see below) – Centralised analysis by RNAseq

- **METALS – Establishing a starting point for iGLOBALS – establish standardised iPSC protocols to produce motor neurons**

  Key Tasks;
  - Perform meta-analysis – could outsource to commercial entity

PITCH

- **Why**
  - Very few examples beyond tofersen of transforming biological knowledge into treatments – need better standardised tools
- **How**
  - iGLOBALS iPSC challenge - select 10 iPSC patient and control lines which we know work in some labs, divide them all over the world
  - Deep analysis of each line in different labs
  - Provide standardised protocols how to analyse lines and analysing results from studies on these lines
- **Success**
  - More drugs progressing through clinical trial
  - More treatments
  - Inclusion for all regions
- **Why Us/Why Now?**
  - Would enable treatments for sporadic disease for the first time
  - Have the knowledge across the globe but this is an opportunity to bring it all together

UNDER-REPRESENTED REGIONS

- **Network Sustainability – Continue building and expanding networks in association with local PLEx**

  Key Tasks;
  - Empower regional network leaders through support (e.g. admin, website etc)
  - Expanding awareness amongst the different networks
  - Including key stakeholders in the conversation

- **SEED Centres – To establish the key seed centres in each under-represented network – establish registries and biorepositories**

  Key Tasks;
  - Identify at least two seed centres in the network (ELATAM/ALSAfrica-Net/ PACTALS)
  - Ensure that each lead centre is inclusive of as many countries that is feasible
  - Build registry(s) and biorepository(s)
  - Deliver education and training (fellowships/mentoring)

- **Super-Grant – Create a grant opportunity specific for under-represented networks – need $$ for grant**

  Key Tasks;
  - International Alliance Research Directors Forum (RDF) to contact each of network leads to identify priority areas
  - Develop grant programme and call for funding applications

PITCH

- **Why**
  - A program grant that will supercharge the under-represented regions that hold 50% of the global ALS/MND population
- **How**
  - Use the Roundtable FTE to develop this program
  - Coordinate SEED centres in the network
  - Build registry and biorepository
  - Deliver education and training
  - Leverage further funding from the RDF for a SUPER grant
- **Success**
  - Under-represented regions now represented in global studies
  - New networks

- Better informed patients
- Better care
- Identification of new potential therapies from new discoveries
- Better access to treatments/equity
- Truly global

- **Why Us? Why Now?**
  - Networks are there – they need to be empowered/resourced
  - We need to know more – biology/ treatments/biomarkers – under-represented regions = fantastic resource

PRECLINICAL EVIDENCE FOR CLINICAL TRANSLATION

- **ACCESS – Enable access to data, models, expertise (translational and research) across academia and industry**

  Key Tasks;
  - Utilise the "Advisory Panel" to enable parties to access data, models, expertise etc to fill in gaps in data and/or knowledge to accelerate translation
  - Consider future use of research outcomes at the start e.g. allow industry access/ commercialisation, IP, consents, contracts, FTO

- **ADD ALS – Access to a pre-clinical advisory board and platform – provide free/fee-for-service advice about pre-clinical models and ideal package**

  Key Tasks;
  - Setting up advisory board – VC/ academics/industry – via call out to ALS/ MND community
  - Streamline communication framework
  - Consider fee-for-service/pre-clinical contract
  - Operational planning – meeting schedule/package guidelines/reviewers' responsibilities
  - Consider set-up of "platform" pre-clinical service

- **Biomarker Use – promote use of biomarkers and appropriate context of use**

  Key Tasks;
  - Promote development of biomarkers during research and discovery
  - Advise on target-engagement biomarkers
  - Advocate and promote data-sharing

- **Existing Drugs – Increase testing of existing drugs as well as new chemical entities, in preclinical studies i.e. new indication for current drugs – short cut into clinical studies**

  Key Tasks;
  - Screening of candidate drugs I animal and other models
  - Review of possible candidates
  - Identify appropriate target engagement and disease biomarkers
  - Validation of mechanism of action
  - Optimisation of compound
  - Patent Search
  - Funding

- **IAF – Industry-Academic Forum – enhance and accessible industry-academic engagement forum**

  Key Tasks;
  - Regular meetings and focus groups
  - Fellowships (3-6 month in industry)
  - Gather appropriate stakeholders
  - Grants to build pre-clinical packages
  - Applying white paper to studies

- **Opinion piece/white paper – outlining broad principles/recommendations for good preclinical development in ALS/MND – to improve understanding of scientific expectations/requirements for preclinical drug development and to build consensus across the field**

  Key Tasks;
  - Align on key principles that should be included in paper
  - Review literature
  - Draft manuscript
  - Send for review to KOLs
  - Present findings at conferences
  - Build engagement/implementation through funders/journals/KoLs

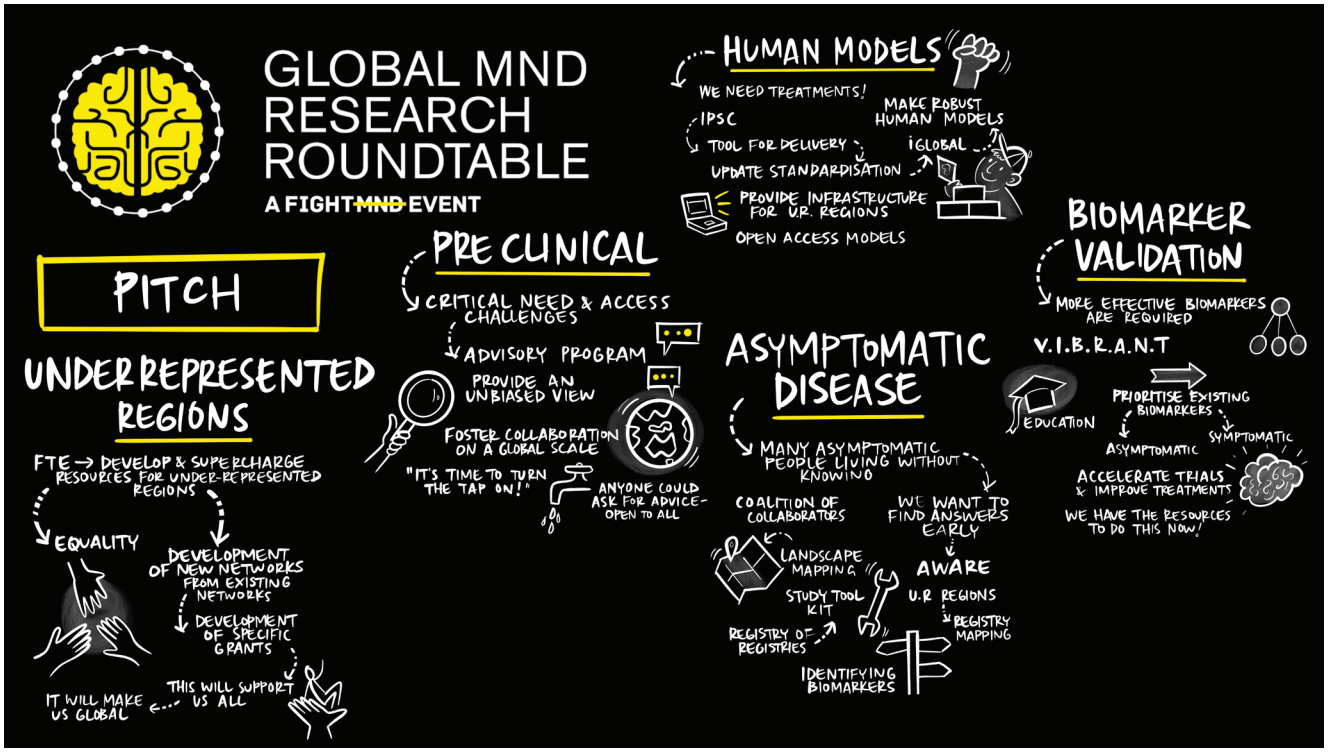
PITCH

- **Why**
  - Translation from preclinical to clinical studies problematic evidence by our lack of successful therapies – 30 years of failure

- **How**
  - TAP – Therapeutic Accelerator Panel/ Program
    - Advisory panel/program in combination with white paper on best practice

- **Success**
  - Better drugs
  - Strengthening preclinical packages
  - Increased collaboration

- **Why Us? Why Now?**
  - Capitalising on advances in field – lots of drugs being developed in preclinical stages
  - Leveraging global talent and expertise
  - Turn on the TAP!!



Following each pitch, the audience were given an opportunity to critique and request further clarification. In the general discussion that followed, there was wide agreement that all the priorities and pitches had strong merit.

Earlier in the Roundtable some attendees expressed doubts as to whether all five priority areas were equally critical to pursue. The work done by the teams over the two days to demonstrate the value of each priority had shifted the mindset of the room to consensus that they should all be further explored.

Throughout the two days, there was a strong sentiment in the room that each of the priorities should maintain consideration of how to develop them with under-represented regions in mind.

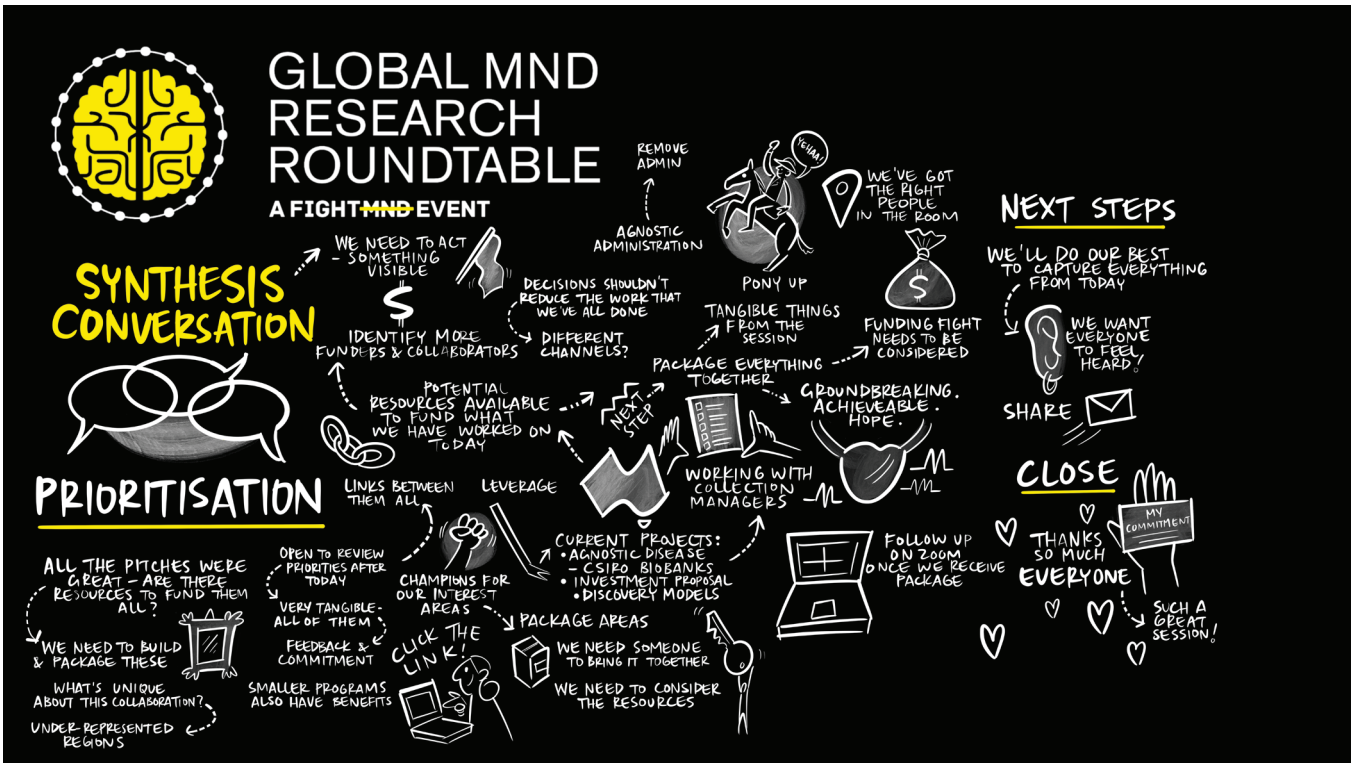
FINAL SESSION: BRING IT ALL TOGETHER – SYNTHESIS CONVERSATION

At the end of two incredibly productive days, we all sat down for a final conversation about where we were at and where we should go next.

- All pitches/priorities were considered important and valuable - how can they be combined/integrated?
- Pitches lose some granularity of the great work that has been done and the value of each priority
- Need to make sure that we're not going to sit here together next year with similar questions
- Need visible outcomes/change the disease
- Lots being done in the UK but at smaller scale and locally
- Outcomes report will provide more granular detail on pitches/priorities
- How to prioritise which outcome first once all the information collated and distributed?
- How can we use our resources to get all pitches moving forward?
- Need to package pitches to leverage further funding
- Not everything needs to be done within Roundtable – some tasks can be done by other groups perhaps already running something similar but at a smaller scale
- Unique thing about this collaboration is under-represented groups – needs to be incorporated into all priorities
- Need to consider how the priorities/pitches can fit in with existing infrastructure
- Need to seek further funding
- For ambitious outcomes need more resources, or limit outcomes
- What can the Research Directors Forum of the Alliance possibly support with?
- Need to make sure outcomes are packaged well – "groundbreaking" "achievable"- message of hope

to ALS/MND community – get them behind us and help support/raise $$

- Do we need an "agnostic" admin core to smooth data sharing/sample collaboration/shared finances/regulatory challenges?
- Could present each pitch/priority as a grant application – work on it to make them as compelling as possible
- Should we meet again virtually to refine the outcomes?

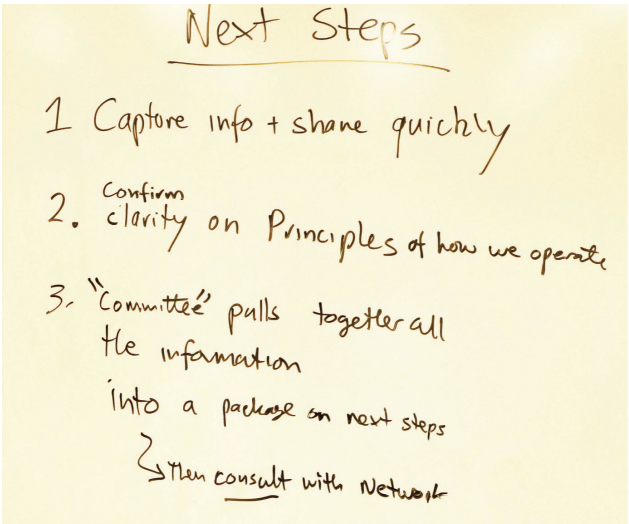


NEXT STEPS

- We will capture all important details and send out for comment
- Decide on next steps – need a management committee

PROPOSED NEXT STEPS

- Five clear priorities were identified through global engagement prior to the Roundtable and the discussions at the Roundtable developed initiatives that will enable these priorities to be advanced. Distillation of the notes and discussions from the Roundtable has presented the following priorities and initiatives. Each of these will be undertaken according to the Vision, Mission, and Principles of the Roundtable as well as ensuring our Strategic Enablers are incorporated. More detail on how these will progress can be found in Appendix 3.



2025 ALS/MND GLOBAL RESEARCH ROUNDTABLE PRIORITIES AND INITIATIVES

Global acceleration of clinical and preclinical biomarker research	Global approach to studying asymptomatic disease	Global best practices and validation of preclinical human ALS/MND models	Global support for enhancing research capacity in underrepresented region	Global best practices for successful
Establish the **VIBRANT** Working Group - **V**irtual **I**nternational **B**iorepository **A**lliance **N**e**T**work consisting of biomarker & biosample repository leaders. **Three step action plan:** 1. Create VIBRANT sample guidelines (including SOPs, data sharing frameworks etc) 2. Locate and provide centralized information to existing samples meeting VIBRANT criteria 3. Prioritize biomarkers and associated context of use for replication and validation work using VIBRANT samples **Additional initiatives:** • BEAST – Biomarker Education and Scientific Training • International scientific exchange program • Selection Committee	Establish the AWARE Working Group – ALS Worldwide Asymptomatic Research Endeavour consisting of willing leaders of existing asymptomatic research cohorts **Three step action plan:** 1. Create a landscape map of existing asymptomatic cohorts (registry of registries) 2. Establish areas of data linkage and propose replication experiments (Mega-analysis) 3. Collectively establish an advisory mechanism for future studies (study toolkit) including best practices for approaching individuals at higher genetic risk and enhancing reach/ participation **Additional initiatives:** • Navigator mechanisms • To connect individuals at high genetic risk to studies/ support groups/clinicians • Education initiatives specifically related to participation in asymptomatic disease research	Establish the iGLOBALS Working Group consisting of ALS-relevant primary human cell model experts **Two step action plan:** 1. Curate a panel of ~10 iPSC lines to establish variability across labs and protocols (standardisation to benchmark iPSC work) 2. Perform meta-analysis with centralised analysis by RNA-seq **Additional initiatives:** • Replication work • Potential for replication of key discoveries within the Working Group or discussion on strategies for effective replication	Establish the Global SEED Working Group – Global South Engagement to Enhance Diversity in ALS/MND consisting of leaders from Global South countries with research or near research capacity **Two step action plan:** 1. Create an annual funding opportunity, competitive only to Global South researchers targeting building capacity in under-represented regions 2. Regular gathering of Working Group for knowledge exchange and potential further action (network sustainability)	Establish the TAP Working Group – Therapeutic Accelerator Panel consisting of diverse experts with broad representation from across Consortia, industry, regulatory, academic and patient communities. **One step action plan:** 1. Evaluation of preclinical packages and recommendations to improve for greater chance of success in clinical trials **Additional initiatives:** • ACCESS - Enable access to data, models, expertise across academia and industry • Opinion piece/white paper

APPENDIX

APPENDIX 1 - GLOBAL ROUNDTABLE ENGAGEMENT PROPOSED MELBOURNE SUB-INITIATIVE GROUPING AND JUSTIFICATION

APPENDIX 2 - SCOPE, OBJECTIVES AND GIVENS (SOGS)

APPENDIX 3 - MND GLOBAL RESEARCH ROUNDTABLE INITIATIVE NEXT STEPS

APPENDIX 1 – GLOBAL ROUNDTABLE ENGAGEMENT PROPOSED MELBOURNE SUB-INITIATIVE GROUPING AND JUSTIFICATION

GLOBAL ROUNDTABLE ENGAGEMENT PROCESS – PROPOSED MELBOURNE SUB-INITIATIVE GROUPING AND JUSTIFICATION

Using the results of the eleven global engagement Zoom calls, we have rolled up the top twelve ideas into five key sub-initiatives, **intended as a starting point** for discussions at the 2025 Global MND Research Roundtable meeting in Melbourne.

Through these actions, all the original work from 2024, including aspects of all seven ambitious goals, will have influenced aspects of this 2025 Melbourne Roundtable work, and each of the engagement call topics are incorporated in some way. Some of them are combined across groups – BIOMARKERS & CLINICAL TRIALS, PRECLINICAL MODELS, HUMAN ALS/MND MODELS & CLINICAL TRIALS.

There were 51 ideas/actions put forward and voted on, in total. The final four global calls addressed the top 18 ideas/actions ranked from the initial seven calls, but also included a Rescue Poll, where individuals would be able to vote for anything from the bottom 33 to be considered further. We have included those six ideas that gathered at least 25% of Rescue Poll votes as "Potential considerations" in the appropriate places below.

Sub-initiatives 1-3 represent actions where globalization can increase POWER & IMPACT

Sub-initiatives 4 & 5 represent actions that require a GLOBALLY COLLABORATIVE EFFORT to be impactful.

1. CREATE AN INITIATIVE TO PRIORITIZE VALIDATION OF ALS/MND BIOMARKERS

a. Including a strategy for timely incorporation into ***better subgrouping for clinical trials***

b. Potential consideration of a ***focus on getting people into trials earlier***

c. Working Group (WG) contributors for consideration would include;

- Individuals who put their name forward for **BIOMARKERS & CLINICAL TRIALS**
- Key trial clinicians with diverse influence

JUSTIFICATION FOR INCLUSION AS FINALIST IDEA FOR MELBOURNE

- ***Create a biomarker validation initiative*** ranked #1 of 51 ideas, with 36% being 10/10 and 73% of scores at 8 or above.
  - This was also the clear leading idea in the **BIOMARKERS** call.
- ***Initiative to focus on better subgrouping of participants and progression subtypes*** ranked #2 of 51 ideas, with 71% of scores at 8 or above.
  - This was also the clear leading idea on the **CLINICAL TRIALS** call.
- Biomarkers will be key to subgrouping in clinical trials so there could be an integration where biomarkers that get field "validation" (for example, NfL as a group-level prognostic biomarker is close, if not there) are driven to include in clinical research/trial studies moving forward.
- **Biomarkers & patient stratification and classification** were two of the key barriers identified in at the outset of the first

PROPOSED GRANULAR WORKING GROUP (WG) ACTIONS

- Working group scoring & prioritization initiative followed by focused efforts on each biomarker – what is needed to achieve "validation", who is not convinced of its use and what would it take to convince or unconvince those who support. (low-cost impact)
- Would include biomarkers for subgrouping participants in clinical trials
- An expanded effort of a forthcoming, European-based initiative where there is a concerted effort to study and evaluate biomarkers in real-time (high-cost impact) – perhaps other countries could focus on other biomarkers under an identical protocol or have independent replication cohorts.
- An additional idea from the RESCUE POLLS that received 27% of the vote, was a focus on getting people into trials earlier, which could be looked at more closely by a WG. Independent and globally collaborative strategies for more timely diagnosis, through addressing healthcare systems, are being worked on but this would conceivably relate more to how science can support these efforts in support of research.

A global effort here increases the POWER over regional studies that can be and are being done.

2. CREATE A GLOBALLY COLLABORATIVE ASYMPTOMATIC ALS/MND INITIATIVE

a. Start with a high-level ***registry of variants being followed***

b. Work towards development of a ***minimum common dataset***

c. Gauge evolving ability to connect existing initiatives and datasets as collaboration grows

- Potential consideration of ***novel approaches to finding initiating factors for "sporadic ALS/MND, studies of unique familial cases (e.g. twin studies, phenocopy studies, etc.) and work to identify genetic modifiers of ALS/MND***

d. Working Group contributors for consideration would include individuals who put their name forward for **ASYMPTOMATIC ALS/MND**

JUSTIFICATION FOR INCLUSION AS FINALIST IDEA FOR MELBOURNE

- ***Create a globally collaborative asymptomatic ALS/MND initiative/consortium*** ranked #4 of 51 ideas after all votes were considered. It also had an average score of 7.6 on the **ASYMPTOMATIC ALS/MND** call – any aspect of the other ideas would essentially fit under this (common dataset, registry), so if there was appetite, it could start with simple collaboration and group.
- ***Establish a minimum common dataset for asymptomatic ALS/MND*** ranked #3 of 51 ideas after all votes were considered and ranked #1 of 7 ideas on the **ASYMPTOMATIC ALS/MND** call with a score of 8.3.
- ***Create a global registry of individuals at high genetic risk being monitored*** ranked #10 of 51 ideas after all votes were considered and ranked #2 of 7 ideas on the **ASYMPTOMATIC ALS/MND** call with a score of 7.9.
- The overall score was pulled down by 9 votes of 3 and below. This could be due to hesitation revealed on the **ASYMPTOMATIC ALS/MND** call around the details of such a registry, that was reduced when considered high-level and with concerns (like privacy) well addressed.
- The **RESCUE POLLS** indicated that amongst the different seven areas, the appetite was highest around globally collaborative efforts for **ASYMPTOMATIC ALS/MND**. This was also the topic with the greatest initial interest, with over 100 individuals signing up to be involved. This work would also help to address the idea to ***focus on getting people into trials earlier.***
- This action would address some aspect of ***all five*** of the key barriers identified in at the outset of the first Roundtable meeting.

PROPOSED GRANULAR WORKING GROUP (WG) ACTIONS

- Use the global reach created through the Roundtable engagement process to locate all individuals who are studying individuals at higher genetic risk of ALS/MND. Ask if they would consider providing the data they collect and the number of each variant they are following, plus any other key data the WG deems appropriate. Put this into a central database that is well curated and shared. The WG could develop a mechanism to create that minimum dataset and recommendations to ensure everyone collects those parameters in addition to anything unique. Additional consideration for maintaining/updating etc. should be considered. **(low-cost impact)**
- Depending on the ease and appetite for collaboration on the dataset standardization and registry, an attempt could be made to create a global umbrella for the collaborative effort, like Project MinE (in optical concept only), to better achieve funding and unite on something that will likely be underpowered everywhere without collaboration. **(high-cost impact)**
- Additional ideas from the **RESCUE POLLS** with at least 25% of the vote, for potential consideration, include novel approaches to finding initiating factors for sporadic ALS/MND (37%), studies of unique familial cases (e.g. twin studies, phenocopy studies, etc.) (29%) and work to identify genetic modifiers of ALS/MND (25%).

A global effort here increases the POWER over regional studies that can be and are being done.

3. CREATE A GLOBAL HUB FOR MORE EFFECTIVE USE OF HUMAN ALS/MND MODELS

a. Including a ***central resource of publicly available, validated IPS lines***

b. Including an ***academic or CRO-based independent validation network***

c. Potential consideration of ***creating best practice recommendations for the creation of human ALS/MND models***

d. Working Group contributors for consideration would include who put their name forward for **HUMAN MODELS** WG

JUSTIFICATION FOR INCLUSION AS FINALIST IDEA FOR MELBOURNE

- ***Create a central resource of publicly available, validated iPS lines & associated data*** ranked #5 of 51 ideas when all votes were considered. This had a score of 7.7 on the **HUMAN ALS/MND MODELS** call.
- ***Setup a network of labs as validated sites/CRO for testing in human models and reproducibility of results*** ranked #9 of 51 ideas after all votes were considered. This ranked #1 and had a score of 8.1 on the **HUMAN ALS/MND MODELS** call.
- The **RESCUE POLLS** indicated that c***reate best practice recommendations for the creation of human ALS/MND models*** was also well considered (27%), with a tie for the third most votes. As this was the third of three ideas, it is safe to conclude that there is a strong appetite to focus on better use of human ALS/MND models, which was reinforced by the **HUMAN ALS/MND MODELS** call discussion.

PROPOSED GRANULAR WORKING GROUP (WG) ACTIONS

- Use the global reach created through the Roundtable engagement process to locate all the iPS lines (participant-derived and created variants, isogenic controls, etc.). WG to help determine what information would be required to gather and how it could be presented effectively in a shared database for global access. **(low-cost impact)**
- The WG could discuss the pros/cons of an academic vs. CRO-based validation mechanism and buil0d a proposed framework for funding consideration. **(high-cost impact)**
- Based on the **RESCUE POLLS**, a best practice recommendations mechanism for the creation of human ALS/MND models (27%) could potentially be considered.

A global effort here increases the POWER over regional studies that can be and are being done.

4. CREATE A STRATEGIC INITIATIVE TO INCREASE GENETIC DATA & BIOSAMPLING FROM UNDERREPRESENTED GROUPS

a. Including a ***best practices mechanism*** to ensure interoperability of all new collection

b. Informed by a ***globally collaborative data dictionary for collection in ALS/MND that includes consideration of individual types of data (-omics, imaging, wearables, etc.)***

c. Potential consideration of ***development of a standard template agreement for biosample sharing and organization of a global summit to discuss big data and biorepositories***

d. Working Group (WG) contributors for consideration would include;

- Individuals who put their name forward for **BIG DATA & BIOBANKING** WGs
- geographical diversity and genetics/biosample experts

JUSTIFICATION FOR INCLUSION AS FINALIST IDEA FOR MELBOURNE

- ***A strategic initiative to increase biosampling of underrepresented groups*** ranked #7 of 51 ideas and scored a 7.6 on the **BIOBANKING** call.
  - The rank went from #4 of 5 ideas on the **BIOBANKING** call to #7 of 51 overall when engaging researchers from a wider diversity of countries.
  - During discussion on the **BIOBANKING** call, a suggestion to prioritize genetics over other biosampling efforts was highly regarded and there was consideration of going back to a revote as the 7.6 may have been higher if more focused on whole genome sequencing.
- ***Best practice SOPs for biorepository collection*** ranked #6 of 51 ideas and was ranked #1 of 5 ideas on the **BIOBANKING** call.
  - Support for reduced siloing of new collections in underrepresented regions provides good reason to create SOPs that could also have impact in regions where collection is already taking place.
- ***A globally collaborative data dictionary for collection of patient measures*** ranked #8 of 51 ideas when all votes are considered. It ranked #1 of 7 ideas with a score of 8.0 on the **BIG DATA** call.
- ***Targeted, global centralization of individuals types of data (-omics, imaging, wearable data, etc.)*** ranked #11 of 51 ideas when all votes are considered. It ranked #3 of 7 ideas on the **BIG DATA** call.
  - This could presumably fit within a data dictionary effort.
- Underrepresented regions would be the **main driver** to do the work but the mechanisms for stronger collection/interoperability of samples/data would have **field-wide impact.**

PROPOSED GRANULAR WORKING GROUP (WG) ACTIONS

- Use the global reach created through the Roundtable engagement process to create a prioritized list of regions/countries/clinics where biosampling is ready to go with investment, through to those that are close, but not there yet, and those that are far away and need a long-term strategy. **(low-cost impact)**
- Develop a mechanism (document or something clever & living) for best practices in biosample and data collection to reduce siloing of new initiatives. A thoughtful mechanism to push for these to be embraced and used would need to be considered. **(medium-cost impact)**
- Work with established mechanisms (Project MinE, ALS Compute, Target ALS, NYGC, etc.) to build a strategy for expansion to underrepresented regions for lowest cost genetics information gathering with open sharing and collaboration. **(high-cost impact)**
- Based on the Top 18 ideas and the **RESCUE POLLS** other ideas that could be considered include development of a standard template agreement for biosample sharing (25%) and organization of a global summit to discuss big data and biorepositories (ranked 13th overall).

This work could not and would not likely be done by regional efforts alone.

5. CREATE A GUIDANCE FOR THE FIELD ON PRECLINICAL EVIDENCE NEEDED FOR EFFECTIVE CLINICAL DEVELOPMENT

a. Potential consideration of best practice recommendations for the creation of human ALS/MND models, developing guidance or harmonized key metrics on reporting of trial results, emphasizing target engagement and biomarkers for translation in pre-clinical research and organization of global versions of successful NEALS/TRICALS/PACTALS initiatives

b. Consideration of people who put their name forward for PRECLINICAL MODELS, HUMAN ALS/MND MODELS & CLINICAL TRIALS

c. Working Group (WG) contributors for consideration would include key clinician-scientists, trial clinicians, animal and human model experts for a multi-faceted WG

JUSTIFICATION FOR INCLUSION AS FINALIST IDEA FOR MELBOURNE

- ***Guidance for industry and clinicians on effective preclinical evidence needed for clinical development*** ranked #12 of 51 ideas when all votes are considered. It was a clear #1 rank out of 8 ideas, with a score of 7.9 on the **PRECLINICAL MODELS** call.
- This action encompasses the #2 and #3 ranked ideas on the **PRECLINICAL MODELS** call, which focused on better translational efforts including **emphasizing target engagement** and **preclinical evidence that led to negative clinical trials.**
- It also addresses several of the ideas put forward on the **CLINICAL TRIALS** call, including ***form a committee for prioritization of therapeutic targets, create a global guidance mechanism for industry and a mechanism/committee for grading preclinical evidence & trials/scorecard.***
- **Better and more effective translation of preclinical data to clinical trials** was a common theme across calls for **PRECLINICAL MODELS, HUMAN ALS/MND MODELS** and **CLINICAL TRIALS.**

PROPOSED GRANULAR WORKING GROUP (WG) ACTIONS

- Create a set of recommendations (document or clever, living innovation) or scoring system for preclinical evidence prior to translation of targets and treatments for ALS/MND. Thoughtful considerations should be made to evolve the recommendations based on trial outcomes, including a retrospective analysis of learnings from negative trials and preclinical evidence that informed them. Could provide a very valuable bridge in the current preclinical/clinical evidence gap that exists. **(medium-cost impact)**
- Based on the Top 18 ideas and the **RESCUE POLLS** other ideas that could be considered include best practice recommendations for the creation of human ALS/MND models (27%), emphasizing target engagement and biomarkers for translation in pre-clinical research (25%), developing guidance or harmonized key metrics on reporting of trial results (27%) and organization of global versions of successful NEALS/TRICALS/PACTALS initiatives (e.g. TRICALS Masterclass format with global attendees) (ranked 15th overall).

This could not and would not likely be done by regional efforts alone.

APPENDIX 2 – SCOPE, OBJECTIVES AND GIVENS (SOGS)

The Scope, Objectives and Givens (SOGs) form the backbone, or the structure of the event. The SOGs are co-designed and developed with the Sponsor Group and the facilitation team over the course of the Sponsor Process and aim to frame the event for session participants.

SCOPE

This statement, or set of statements, describes the focus of the session. Elements that may be referenced in a statement of scope are: topics, timing, functions, geographies, or demographics (to name a few). A good scope statement is a noun phrase and shouldn't start with a verb.

Accelerating research impact through global collaboration and alignment.

OBJECTIVES

This is a series of statements that defi ne what the group needs to have achieved by the end of the event. Good objectives are concise, ambitious and expressed as verb phrases. Together we will:

- Review the progress made to date since the inaugural Roundtable
- Broaden our perspectives through including people with lived experience and experts from other relevant domains.
- Get up to speed with the current state of play and key challenges across the 5 priority areas:

  Biomarker validation
  Asymptomatic disease
  Human models
  Underrepresented regions
  Preclinical to clinical development

- Assess the state of play, review and prioritise the critical challenges to solve, and explore what success looks like across each of these priority areas.
- Explore incentives and barriers to global collaboration from the perspective of roles represented in the room and key external stakeholders.
- Experience the realities of collaboration through a hands-on challenge, and refl ect on what we learn together.
- Develop high-level approaches to address the critical challenges, including actions, quick wins, timing, interdependencies, risks, required capabilities, resources, and responsibilities.
- Refine implementation details, including our shared vision and mission, governance and working group structures, and options for resourcing and funding.
- Reconnect with people and meet new people in the global MND research community.
- Experience new, innovative ways of working in a fun and memorable event.

GIVENS

These statements are design parameters, and other non-negotiables, for the Event. They should inform and/or support the Scope and Objectives.

- While care, access to care, and advocacy are critical issues, the primary focus of this Roundtable is research and collaboration.
- These are complex challenges, and past efforts have not always succeeded – so we should approach them with open minds, courage and optimism, and keep a focus on the bigger picture.
- We will build on and complement existing initiatives, avoiding duplication where work is already being done effectively.
- We can't do everything. To stay focused, this workshop will centre on fi ve priority areas identifi ed through working group feedback and discussion. While there is flexibility, these will remain the primary focus for our efforts.
- All information shared here is open and non-confidential

APPENDIX 3 – MND GLOBAL RESEARCH ROUNDTABLE INITIATIVE NEXT STEPS

Five clear priorities were identified through global engagement prior to the Roundtable and the discussions at the Roundtable developed initiatives that will enable these priorities to be advanced. Each initiative will be undertaken according to the Vision, Mission, and Principles of the Roundtable as well as ensuring our Strategic Enablers are incorporated.

VISION

A world without ALS/MND

MISSION

To accelerate research through global collaboration and alignment

PRINCIPLES

We are **COURAGEOUS**
We are **GLOBALLY MINDED**
We value **MEANINGFUL PARTNERSHIPS**
We will **BALANCE COLLECTIVE & INNOVATIVE THOUGHT**
We are **INCLUSIVE & RESPECTFUL**
We work with **URGENCY**
We work **EFFICIENTLY**

KEY STRATEGIC ENABLERS

An engaged **COMMUNITY** of people affected by ALS/MND at every level of governance

A **COMPLEMENTARY** approach to the current landscape to avoid duplication of efforts

COLLABORATIVE resourcing of funding and in-kind contributions

Proposed Leadership Group: Researcher and lived experience co-Chairs from each coalition (determined at or after first meeting) and possibly Bec, Gethin, David

Support: Forthcoming Project/Program Manager to be hired

The five strategic goals are described on the following pages;

1. Create an initiative to prioritize **VALIDATION OF ALS/MND BIOMARKERS**
2. Create a globally collaborative **ASYMPTOMATIC ALS/MND INITIATIVE**
3. Create a global hub for **MORE EFFECTIVE USE OF HUMAN ALS/MND MODELS**
4. Create a strategic initiative to **INCREASE GENETIC DATA & BIOSAMPLING FROM UNDER-REPRESENTED GROUPS**
5. Create a guidance for the field on **PRECLINICAL EVIDENCE NEEDED FOR EFFECTIVE CLINICAL DEVELOPMENT**

GLOBAL ACCELERATION OF CLINICAL AND PRECLINICAL BIOMARKER RESEARCH

- **VIBRANT** Working Group - **V**irtual **I**nternational **B**iorepository **A**lliance **N**e**T**work
- Consisting of biomarker & biosample repository leaders
- Four-step action plan (to start):
    1. *Create VIBRANT sample guidelines*
    2. *Locate and provide centralized information to existing samples meeting VIBRANT criteria*
    3. *Prioritize biomarkers and associated context of use for replication and validation work using VIBRANT samples*
    4. *Create a list of actions/experiments required to validate or de-validate the top prioritized biomarkers*

PROPOSED PATHWAY TO LAUNCH & ACTION:

1. Determine VIBRANT Working Group membership
    - Email all stakeholders engaged to support a biomarker priority area (before and in Melbourne)
    - Request a list of up to ten nominations from everyone (kept anonymous)
    - Considerations highlighted including gender, geography (not heavily weighted towards North America), career stage, diversity, and relevant expertise & knowledge
2. Use nominations to curate invitation list – invite members accordingly
3. Gather working group to discuss first steps (90 min max)

First meeting draft agenda (subject to change – support from Bob Bowser, Mary-Louise Rogers)

Moderated by Bec, Dave and/or Gethin – future meetings via Program Manager

TIME	TOPIC
0-10 min	Welcome & introductions
10-20 min	Provide succinct, relevant background to working group members
20-45 min	Provide four-step action plan for working group and solicit feedback
45-60 min	Consider additional action items raised in Melbourne
60-70 min	Consider additional action items raised in Melbourne • Written VIBRANT guidelines/SOPs or other format? • BEAST (Biomarker Education And Scientific Training) • Data sharing framework? • Search engine/digital tool? • Additional feasible ideas?
70-80 min	Present proposed work plan for first four steps • To be sent out for comments
80-85 min	Brief discussion on the will of the members to continue on & do the work • Are there any key individuals missing from the group?
85-90 min	Establish working group Chair(s), additional details (naming, etc.)
85-90 min	Discuss next steps & next meeting

GLOBAL APPROACHES TO STUDYING ASYMPTOMATIC DISEASE

- **AWARE** Working Group – **A**LS **W**orldwide **A**symptomatic **R**esearch **E**ndeavour
- Consisting of willing leaders of existing asymptomatic research cohorts
- Three-step action plan (to start):
    1. Create a landscape map of existing asymptomatic cohorts (registry of registries)
    2. Establish areas of potential data linkage and explore creation of a replication experiment and mega-analysis pathway
    3. Collectively establish an advisory mechanism for future studies (study toolkit)
        - including best practice sharing mechanism for approaching individuals at higher genetic risk and enhancing reach/ participation

PROPOSED PATHWAY TO LAUNCH & ACTION

1. Determine ideal AWARE Working Group membership
    - Email all stakeholders engaged to support an asymptomatic priority area (before and in Melbourne)
    - Request a list of up to ten nominations from everyone (kept anonymous)
    - Considerations highlighted including gender, geography (not heavily weighted towards North America), career stage, diversity, and current leadership/involvement in an asymptomatic ALS/MND cohort and/or with overlapping FTD participants
2. Use nominations to curate invitation list – invite members accordingly
3. Gather working group to discuss first steps (90 min max)

First meeting draft agenda (subject to change – support from Maria del Mar Amador, Matt Harms)

Moderated by Bec, Dave and/or Gethin – future meetings via Program Manager

TIME	TOPIC
0-10 min	Welcome & introductions
10-20 min	Provide succinct, relevant background to working group members
20-45 min	Provide three-step action plan for working group and solicit feedback
45-60 min	Consider additional action items raised in Melbourne • Navigator mechanism to connect people carrying genetic variants to research opportunities • Additional feasible ideas?
60-70 min	Present proposed work plan for first three steps • To be sent out for comments
70-80 min	Brief discussion on the will of the members to continue on & do the work • Are there any key individuals missing from the group?
80-85 min	Establish working group Chair(s), additional details (naming, etc.)
85-90 min	Discuss next steps & next meeting

GLOBAL BEST PRACTICES AND VALIDATION OF PRECLINICAL HUMAN ALS/MND MODELS

- iGLOBALS Working Group
- Consisting of ALS-relevant primary human cell model experts
- Three-step action plan (to start):
    1. Curate a panel of ~10 iPSC lines to establish variability across labs and protocols (standardisation to benchmark work)
    2. Create a pathway of how to effectively use the chosen lines to reduce heterogeneity
    3. Create a pathway for replication of key discoveries

PROPOSED PATHWAY TO LAUNCH & ACTION:

1. Determine ideal iGLOBALS Working Group membership
    - Email all stakeholders engaged to support a human model priority area (before and in Melbourne)
    - Request a list of up to ten nominations from everyone (kept anonymous)
    - Considerations highlighted including gender, geography (not heavily weighted towards North America), career stage, and relevant expertise
2. Use nominations to curate invitation list – invite members accordingly
3. Gather working group to discuss first steps (90 min max)

First meeting draft agenda (subject to change – support from Jeroen Pasterkamp, Brad Turner)

Moderated by Bec, Dave and/or Gethin – future meetings via Program Manager

TIME	TOPIC
0-10 min	Welcome & introductions
10-20 min	Provide succinct, relevant background to working group members
20-45 min	Provide three-step action plan for working group and solicit feedback
45-60 min	Consider additional action items raised in Melbourne • METALS – meta-analysis • Additional feasible ideas?
60-70 min	Present proposed work plan for first three steps • To be sent out for comments
70-80 min	Brief discussion on the will of the members to continue on & do the work • Are there any key individuals missing from the group?
80-85 min	Establish working group Chair(s), additional details (naming, etc.)
85-90 min	Discuss next steps & next meeting

GLOBAL SUPPORT FOR ENHANCING RESEARCH CAPACITY IN UNDER-REPRESENTED REGIONS

- Global **SEED** Working Group – Global **S**outh **E**ngagement to **E**nhance **D**iversity in ALS/MND
- Consisting of leaders from Global South countries with research or near research capacity
- Two-step action plan (to start):
- Create parameters for an annual funding opportunity, competitive only to Global South researchers targeting building capacity in under-represented regions
- Curate a list of priorities and tangible actions where the ALS/MND ecosystem can work effectively with Global South regions
- Advances toward previous strategic goals on **BIG DATA, BIOBANKING, ASYMPTOMATIC DISEASE, HUMAN ALS/MND MODELS, BIOMARKERS, CLINICAL TRIALS**

PROPOSED PATHWAY TO LAUNCH & ACTION:

1. Determine ideal Global SEED Working Group membership
    - Email all Global South individuals signed up to engage in Global Roundtable Initiative at any step (and those working on this topic in Melbourne)
    - Request a list of up to ten nominations from everyone (kept anonymous)
    - Considerations highlighted including representation of a Global South region, gender, geography, individuals with a good knowledge of hurdles faced
2. Will additionally request for up to three names as potential Global North champions
3. Use nominations to curate invitation list – invite members accordingly
4. Gather working group to discuss first steps (90 min max)

First meeting draft agenda (subject to change – support from Jose Manuel Matamala, Nortina Shahrizaila)

Moderated by Bec, Dave and/or Gethin – future meetings via Program Manager

TIME	TOPIC
0-10 min	Welcome & introductions
10-20 min	Provide succinct, relevant background to working group members
20-30 min	Provide two-step action plan for working group and solicit feedback
30-40 min	Consider additional action items
40-70 min	Discuss plan for development of Global South grant opportunity • Address key questions required to build a draft Terms of Reference • Additional feasible ideas?
70-80 min	Brief discussion on the will of the members to continue on & do the work • Are there any key individuals missing from the group?
80-85 min	Establish working group Chair(s), additional details (naming, etc.)
85-90 min	Discuss next steps & next meeting

GLOBAL BEST PRACTICES FOR SUCCESSFUL PRECLINICAL TO CLINICAL TRANSLATION

- TAP Working Group – Therapeutic Accelerator Panel
- Two-step action plan (to start):
    1. Create framework for a group to evaluate preclinical packages and make recommendations for improvement to achieve greater success in clinical trial
    2. Determine how the group could be effectively implemented and maintained
- Consisting of diverse experts with representation from:
    - NEALS, TRICALS, PACTALS,
    - Industry
    - Patient community
    - Animal model experts
    - Human/iPS model experts (NeuroiPSC representative)
    - ALS pathobiology expert
    - Biomarker experts (VIBRANT rep?)
    - Regulatory affairs (FDA, EMA)
    - CMC etc.

PROPOSED PATHWAY TO LAUNCH & ACTION:

1. Revisit key individuals from Melbourne to build out expertise list for considerations
2. Determine ideal TAP Working Group membership
    - Email all stakeholders engaged to support a priority area (before and in Melbourne)
    - Request a list of up to ten nominations from everyone (kept anonymous)
    - Considerations highlighted including gender, geography (not heavily weighted towards North America), career stage and relevant expertise (using list curated by Melbourne individuals)
3. Use nominations to curate invitation list – invite members accordingly
4. Gather working group to discuss first steps (90 min max)

First meeting draft agenda (subject to change – support from Lucie Bruijn, Keith Mayl)

Moderated by Bec, Dave and/or Gethin – future meetings via Program Manager

TIME	TOPIC
0-10 min	Welcome & introductions
10-20 min	Provide succinct, relevant background to working group members
20-30 min	Provide two-step action plan for working group and solicit feedback
30-40 min	Consider additional action items raised in Melbourne • Mechanisms to promote biomarkers and context of use • Industry-academic forum • Additional feasible ideas?
40-70 min	Present proposed work plan for first two steps • To be sent out for comments
70-80 min	Brief discussion on the will of the members to continue on & do the work • Are there any key individuals missing from the group?
80-85 min	Establish working group Chair(s), additional details (naming, etc.)
85-90 min	Discuss next steps & next meeting

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