

Involving people living & working with MND to innovate, design and develop MND SMART

Patient & Public Involvement Event
12th March 2024

Judy Newton
Motor Neuron Disease Nurse Consultant



THE UNIVERSITY
of EDINBURGH



MY
NAME'S
DODDIE
foundation



MND-SMART

Clinical trials for MND



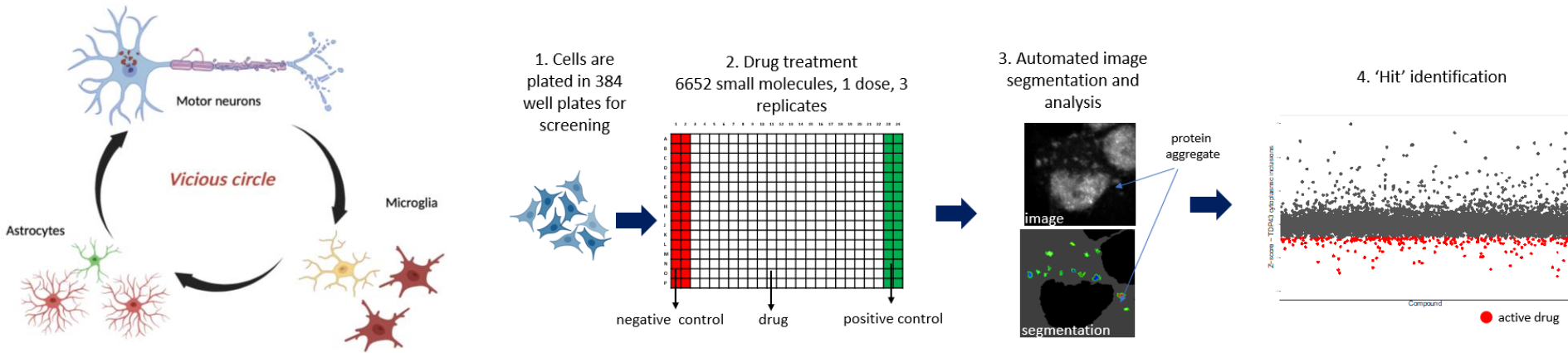
Motor neuron disease

- Rapidly progressive & fatal
- Limb weakness, speech/swallowing, breathing difficulties
- 50% cognitive/behaviour change, 15% dementia
- Time to reach diagnosis: 1 year
- Average survival: 2-3 years, 30% die within 1 year
- c. Lifetime risk 1:300
- Only 1 licensed drug – 1995 – extends life by 2 months
- **< 10% people with MND in trials**

MND SMART (Systematic Multi Arm Adaptive Randomised Trial)

A UK Platform for Definitive MND TRIALS

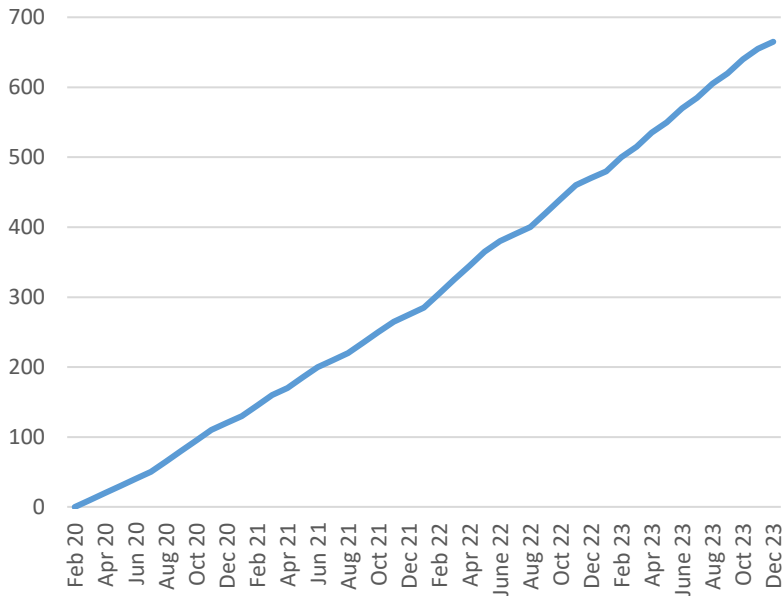
A UK wide inter-disciplinary partnership of experts in MND disease biology, stem cells, drug discovery, innovative trial methodology, & people living with MND



MND-SMART

Clinical trials for MND

MND-SMART Recruitment



Recruitment rate of c.15 participants/month



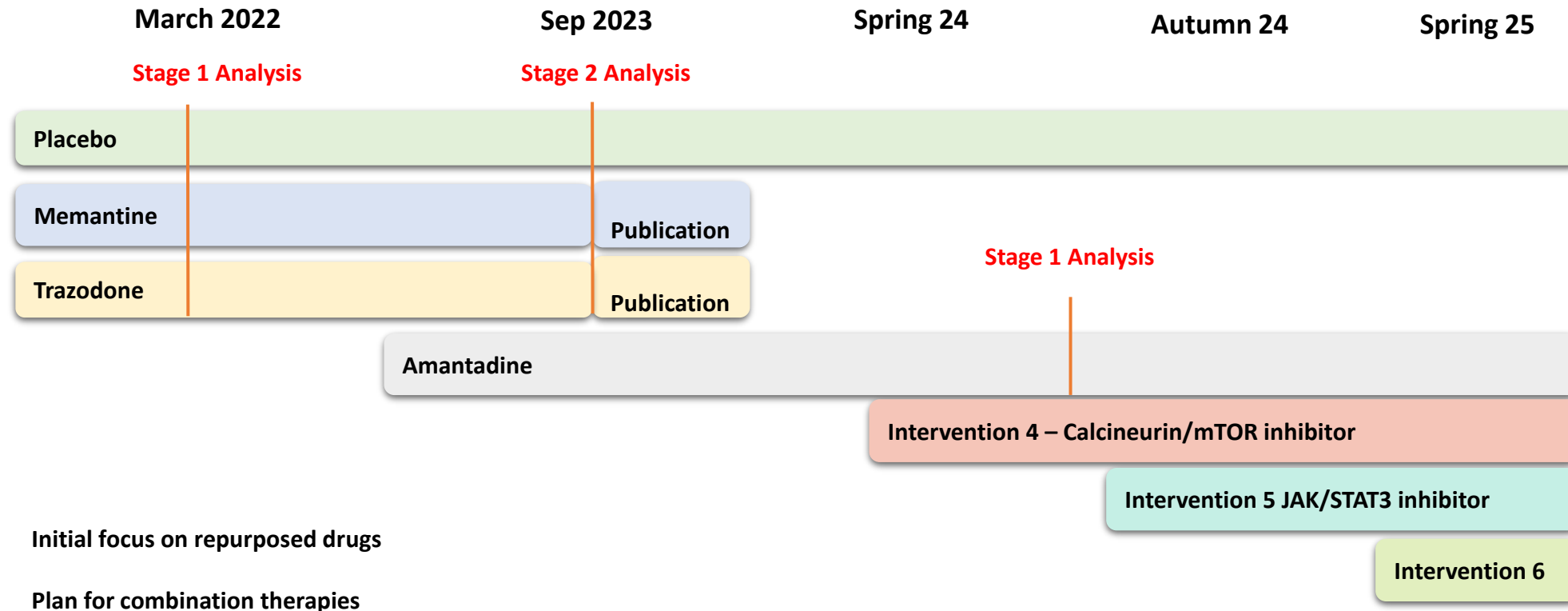
Unprecedented speed and scale

Launch of the randomised trial amidst challenges of Covid-19	2020
Sites in the UK across all 4 nations	23
Randomised participants	>700
Years in operation	4
Interventions conclusively evaluated	2

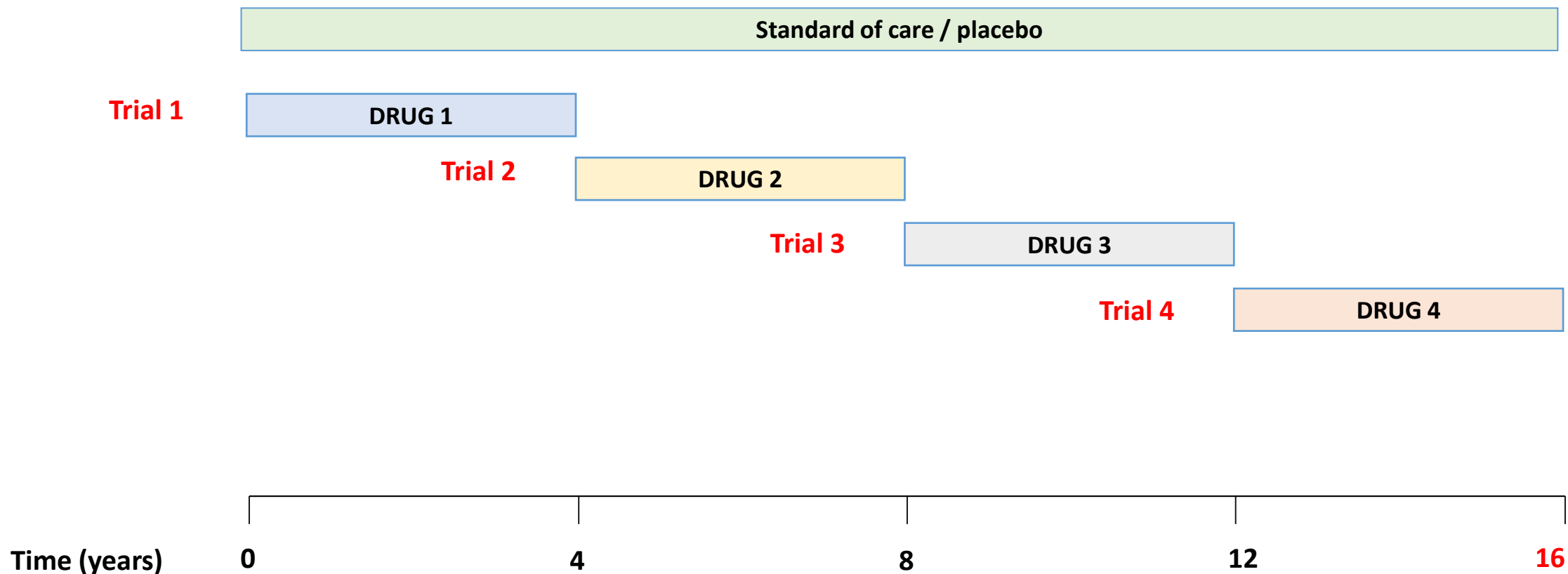
Time compared to conventional trial design

We strive for equity of access for pwMND throughout the UK regardless of location, social status, or ethnicity

MND SMART- Definitive UK Platform for expeditious Phase 3 MND Trials



How long might it take to test 4 new interventions using a standard approach?

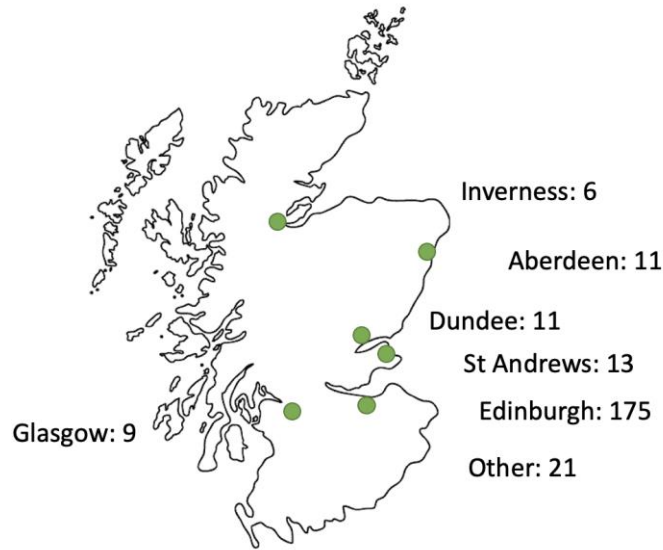


At least 16 years to test 4 interventions

2007 - The Euan MacDonald Centre

Pioneering vital research into motor neuron disease.

Uniting over 200 scientists and health professionals across Scotland.



The mission of the Euan MacDonald Centre is, through research, to improve the outcomes for people living with motor neuron disease (MND) and related conditions.

Who we are

The Euan MacDonald Centre for Motor Neuron Disease Research is based at the University of Edinburgh and is a not-for-profit, charitable network of over 200 researchers across Scotland. We use research to improve the lives of people living with motor neuron disease (MND) and related conditions.

Today, it takes too long to make the diagnosis, there are no truly effective treatments, and clinicians are unable to offer individualised prognosis. The Centre is working in all these areas.

- **A primary ambition of the MacDonald family was to bring MND trials to Scotland**





Key influencers of change -

advised by people affected by MND

We have been

- Led by
- Advised by
- Influenced & crucially
- Encouraged by people directly impacted by MND
- The grouping evolves with the trial

Game changing encouragement for the trial management team

“Nothing is worse than living with MND.

We will participate in trials of any drugs that may work to slow, stop, and reverse this disease – regardless of their potential side effects and the need to monitor for safety”



MND SMART PPIE group member, Jan 2023



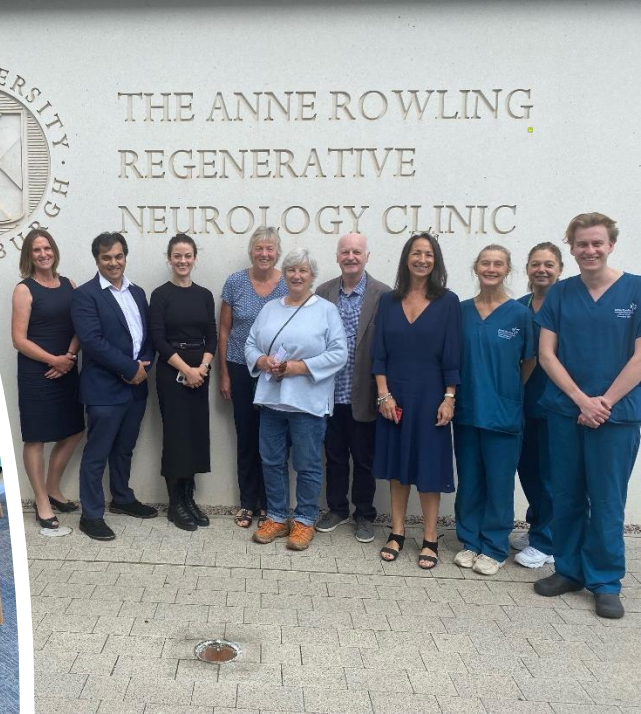
Throughout MND-SMART's history we have worked with PPIE. We asked the PPIE to inform the nation of the interim analysis results

MND-SMART

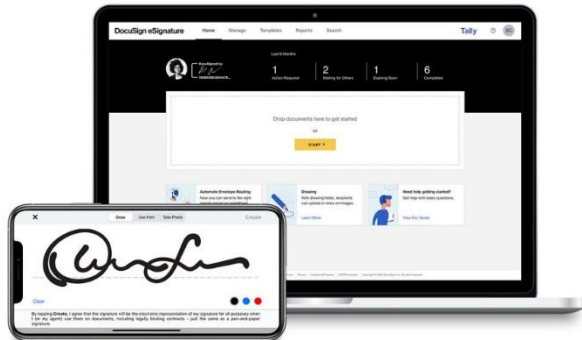
Clinical trials for MND

A UK wide inter-disciplinary team innovating trial methodology in partnership with people working with MND

- Remote assessments ?
- Direct feedback from teams working on the trial – *“can we change the ecrf to auto-populate fields?”*
- *“Can we slow down recruitment to ensure data integrity?”*



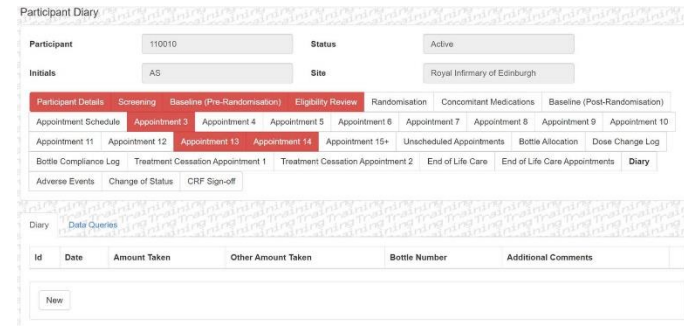
Innovation implemented in MND SMART



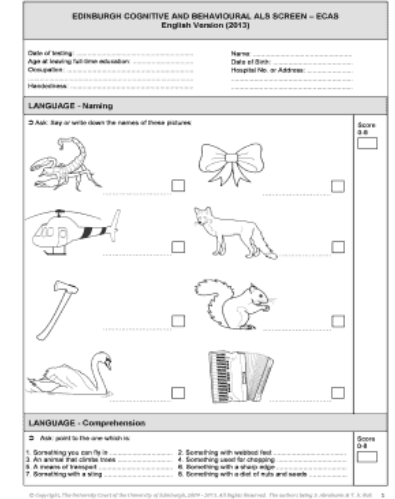
E-consenting



Video conference follow ups



Electronic diary cards



Remote ECAS



Capsule sizes/opening
Of container



Layout of documents. "cut to the chase , don't leave the "but"to the end !



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Getting feedback ...

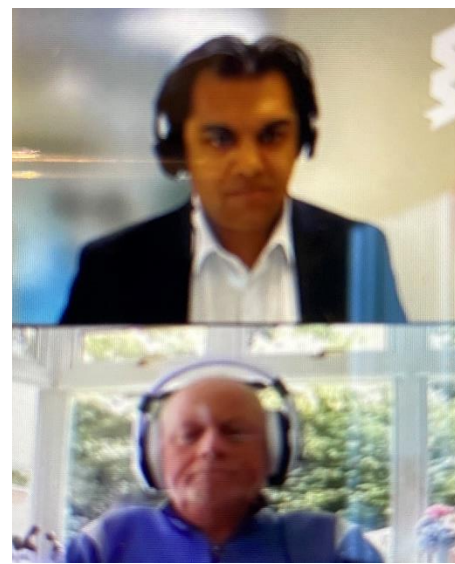
I love the Fast Facts and new IMP flyer plus the colour. One of my Marketing gurus when I was still working told me that colour signifies trust and tranquillity!

No problem, Judith. I was really happy to help out. It was great to see the people who are doing their best to make inroads to a cure one day.

Also, I think the new section in the PIS about the fate of blood samples hits the mark. Regarding the two updated draft PIS documents, I can only echo Stevie's comments about the realistic possibility that a proportion of signatories will not read the documents fully – but also fully understand the need for the comprehensive content & (accordingly) length.

One aspect I do have a query about is the explanation in Section 3 of both Draft v 12 & Draft v11 regarding the new drug, Tacrolimus. Both read:

Is that the correct expression? It seems to me that the word "improve" is out of place



- **Amy many thanks for passing these over for review. I'm happy with the “What happens to my research blood samples”. It is clear and concise. I understand the need for the PIS documents to cover all medical, legal and technical bases. Given they run into a Word count of over 8,000 each, I'm fairly sure some won't read it all however "caveat emptor". It is comprehensive and in plain English.**

Delivering the Christmas wish for 2024

MND-SMART Christmas Wish

Acknowledgements

MND-SMART Participants & Patient & Public Involvement and Engagement group, National MND nursing team, MND SMART research teams across UK, Trial Management Team, Independent Committees, Funders

