The ROADMAP project an EU consortium to improve care in Alzheimer's disease

Introduction

The ROADMAP communications team carried out an interview with members of the Outcome Definition team (WP2), Mia Nelson, Claire Tochel, and Amanda Ly from the University of Edinburgh. This interview features the work they are currently doing in gaining a new look at our understanding of outcomes and their respective importance.

Meet ROADMAP's Outcome Definition team co-leaders

Prof. Cathie Sudlow is Head of Centre for Medical Informatics and Professor of Neurology and Clinical Epidemiology at the University of Edinburgh. She is also Chief Scientist for UK Biobank, an open access, prospective population-based cohort of 500,000 UK adults with comprehensive genotype and phenotype data.

Christin Bexelius is International Payer Strategy Leader and responsible for pricing and market access at Roche. She holds a PhD in medical epidemiology and an MSc in Biomedicine.

Why are you doing research on outcome definitions?

Claire: The research so far trying to develop treatments for Alzheimer's disease (AD) has not fully unfolded its potential in capturing the outcomes that are important to people that live with the disease. It seems to be part of a big movement within Europe and across the world to redress that balance and bring the focus back to working out what's important to people living with the disease. ROADMAP is a big part of addressing this gap.

Amanda: It is important to identify different types of outcomes relevant to AD across its spectrum; we've been looking at a wide range to capture the impact of dementia on people affected and their carers. These include cognitive (for example, the ability to remember something) and functional impairment outcomes (such as being able to drive). In addition, we also consider neuropsychiatric symptoms, impact on the caregiver and significant disease-related events across the life course (such as moving into an institution) just to name a few.

What does your work in the Outcome Definition team involve?

Amanda: At the very beginning, we started with a pragmatic review. This was our first project deliverable. For that, we looked at literature that relates to outcomes and outcome measures relevant to AD. Doing so, we identified 11 categories of outcomes, including cognitive and functional impairment, behavioural and neuropsychiatric symptoms, health and social care costs, impact on the caregiver, biomarkers, as well as other kinds of clinical outcomes, such as comorbidities.

Claire: That first list of outcomes set the scene. It was a broad sweep across currently available information. The systematic review followed from that to focus on three stakeholder groups: patients, carers, and healthcare professionals. We took a systematic process to look for all available evidence on what those groups considered to be important in terms of AD outcomes.

Amanda: We also launched online and paper surveys to identify important outcomes to consider when assessing meaningful change in disease progression. This pan-European survey is an exciting and important aspect of WP2. It involved reaching out to patients and carers as well as professionals that are concerned with dementia in their work. It was great to collaborate closely with our WP2 partners in academia and industry to design the surveys.

Amanda: I'd also like highlight that we had great help in distribution by friends from affiliated projects such as EPAD, EMIF-AD, DPUK, and others. The paper surveys were distributed in memory clinics based in Girona, Oxford and Edinburgh.

Mia: The other activity we are undertaking to answer what the priority outcomes are, are stakeholder engagement interviews and consultations. For this, we carried out consultations with the European Working Group of People with Dementia as well as their carers/supporters respectively family members. In addition, we are conducting individual interviews with various professionals including clinicians, scientists, health economists, ethicists, industry representatives, and advocacy groups.

While the surveys will inform us on priorities, the interviews help us to understand what makes specific outcomes a priority over others. This will give us an insight into the nuances and the details behind the outcomes.

For WP2, each of these activities – the original outcomes list, the systematic review, the surveys, and the interviews and workshops – fit together in pursuing an overarching aim. There is a crucial need to have a good understanding of what's already been done, what people across Europe and the different stakeholders think is a priority and why. We hope to produce a thorough, detailed piece of work at the end, and a good answer.

There are different understandings on what an outcome is, how did you approach this difficulty for the different tasks?

Claire: From the systematic review perspective we've let the literature inform us what an outcome is. We started this task with an open mind without a pre-defined list of outcomes that we were looking for. We used sensitive, general search terms to begin with to capture a broad range of papers, and then applied specific inclusion criteria to narrow our selection down to the right types of studies, the right disease group and the right stakeholder groups.

Amanda: With regards to the surveys, we had the first deliverable to work with; the outcome categories we established were used to inform the structure of the surveys. With our outcome categories, our starting point was outcomes that we traditionally think of in epidemiology - outcomes you might find in clinical trials – but then we expanded our scope given that we work on a real-world data project. In a sense, we are identifying a bridge between traditional outcomes research and RWE research.

Mia: From the workshop and interview perspective, it is a lot in keeping with the systematic review plan, taking a broad approach to it. An outcome should be whatever the participants suggest it is.

Mia: If they for example find something useful to help monitor disease progression, or they state what they consider to be an important part of a meaningful delay in disease progression. The idea with ROADMAP is to take a new look at our understanding of outcomes. It's been important to balance these aspects – knowing what has already been done, what is traditional, what's useful within it, and finding out from more qualitative aspects what has been missed in the past.

How will your team move forward?

Claire: Bringing our different approaches and tasks together is an exciting challenge. Moving forward, this is where the international, pan-organisational input is becoming really impactful. We have experts from across the world and every sector of this area of work, who are all giving advice on how to tackle this challenge. We gathered evidence from different types of sources, and that's already valuable. But now we can see where the same issues come up repeatedly from completely different methods of elicitation. At the end of the day, it is collaboration that is going to add weight to identify what is important.

Amanda: After we synthesise the results on RWE priority outcomes from all WP2 work streams, our work will feed into mapping these outcomes in real-world data sources with WP3 in a gap analysis exercise. Further, we are looking forward to see how outcomes are prioritised at different stages of the disease, since this will be informative for disease modelling, which is being conducted by the disease modelling and simulation team, and for the ROADMAP data cube. We look forward to working closely with other WPs as we progress in establishing a full body of evidence in WP2.