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## Background

The [TREAT-NMD Advisory Committee for Therapeutics](#) (TACT) is a unique multi-disciplinary international group of academic and industry drug development experts, including patients and/or patient representatives. TACT gives advice on the translational and development pathway of therapeutic programs in rare neuromuscular diseases. The voice of the patient is integral to this process, as they can provide their unique perspective on the relevance of the proposed therapy to patients, patient burden and the patient perspective on the disease itself. The patient's needs and the benefit/safety ratio of the therapy under development are the fulcrum of the entire process.

The TACT committee was created to address a critical need to prioritise drug development in neuromuscular diseases, with large unmet needs and limited numbers of patients able to be enrolled into clinical trials.

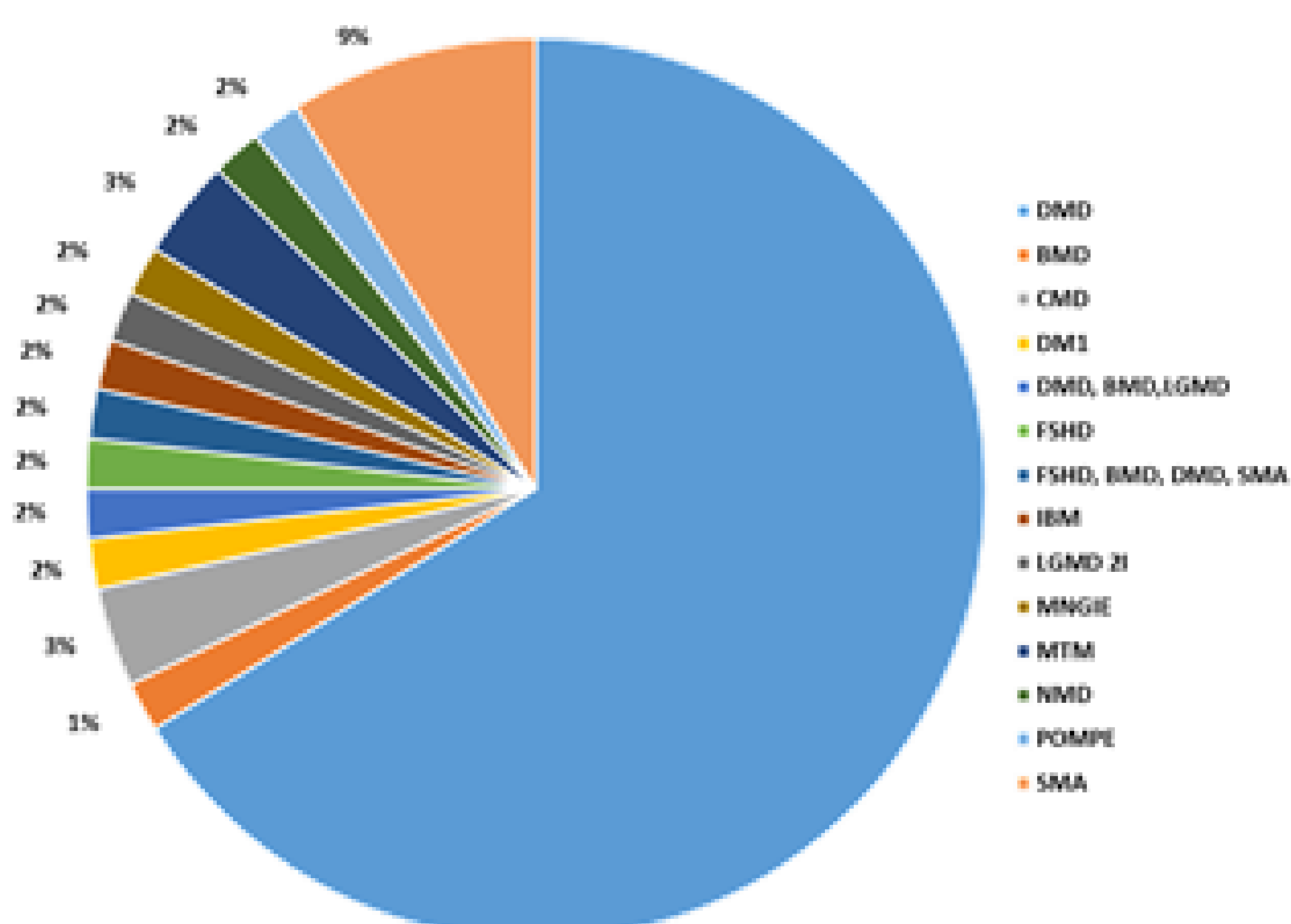
## Aim and method

Due to the success of TACT in the neuromuscular field, a toolkit to replicate the model in other rare disease areas has been produced within the [European Joint Programme Rare Disease](#) (EJP RD)\* project. **The Advisory Committee for Therapeutics (ACT) toolkit is intended to support rare disease networks to establish their own ACT, by providing them with step-by-step guidance and template documentation.** The toolkit will be available, along with other resources designed to support therapy development, through a virtual library to be featured on the EJP RD website later this year.

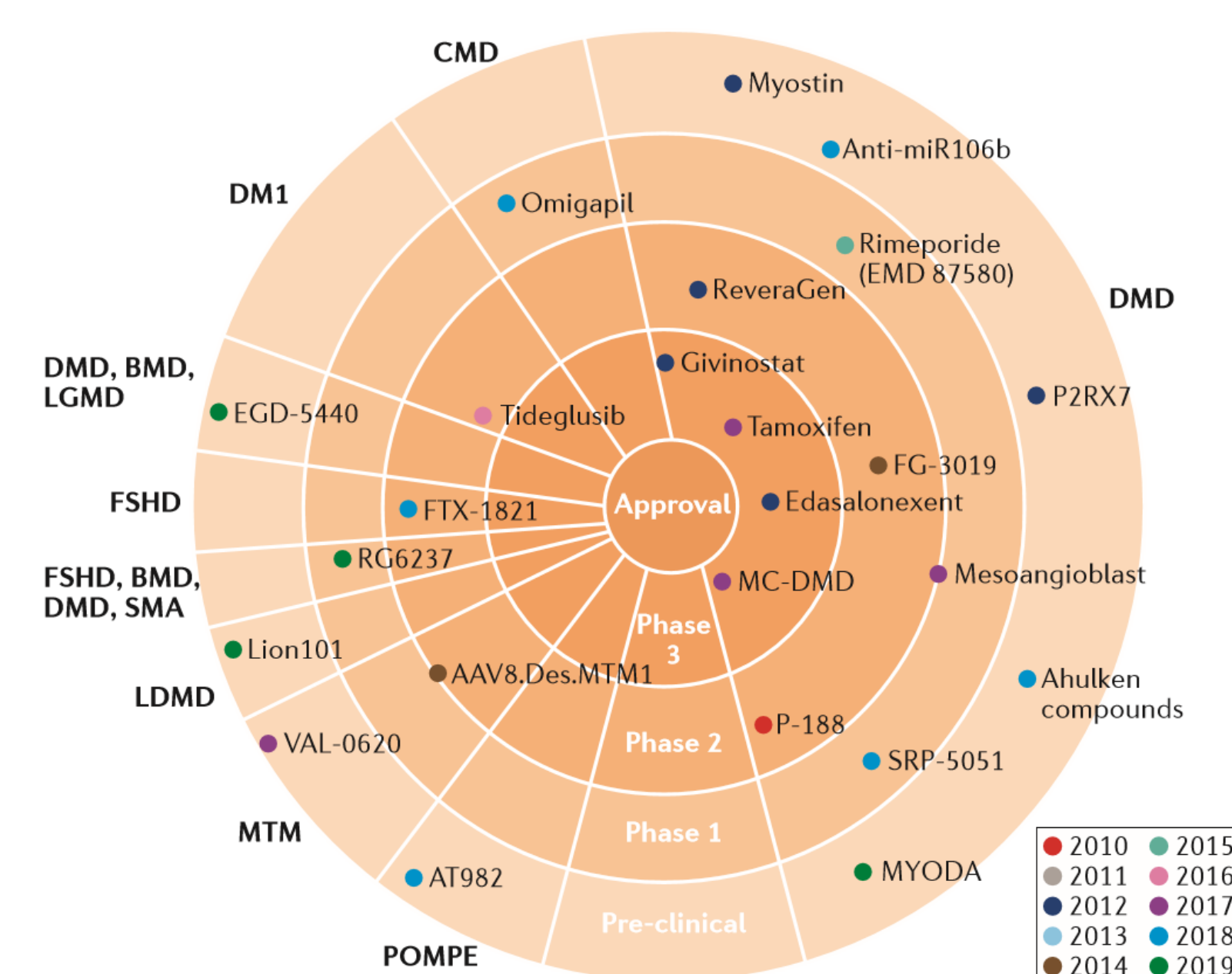
## Results

Our decision to replicate the TACT model in other disease areas is based on the results of TACT, some of which are detailed below. The third image shows a collection of pages from the ACT toolkit.

Reviewed TACT applications by disease area



Reviewed TACT applications still in development



Advisory Committee for Therapeutics (ACT) toolkit



It is important to note that the success of an ACT can be measured by the establishment of a systematic approach, to identify the realistic prospects for a particular compound. Whether the outcome of a review is positive or negative, this approach can foremost benefit patients, as well as the field and the sponsor.

We were recently successful in submitting a proposal to the EJP RD call - [ERN RD Training and Support Program](#), for funding to hold a workshop designed to communicate the benefits of establishing an ACT. We hope that the workshop provides ERN representatives with a forum to explore the utilisation of the ACT model in the ERN domain. The date of the workshop is to be confirmed. If you would like more information, please contact [joanne.lee@newcastle.ac.uk](mailto:joanne.lee@newcastle.ac.uk).

## Conclusion and next steps

The aim of an ACT is to help sponsors position their candidate compound along a realistic and well-informed pathway to clinical trial and eventual registration, by identifying potential pitfalls in pre-clinical data packages and clinical trial design and giving independent and transparent advice. Clinical trials in rare diseases share similar challenges to those in the neuromuscular field including, low patient number, lack of clarity around outcome measure and lack of natural history data. The TACT model itself is readily transferable to many other rare disease areas.