# EURORDIS MRD School 2025

# Designing a patient-centric clinical development programme

**2-hours workshop**

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* **Presentation** of the session, overall objectives, and introduction to the case study + opportunities to ask questions (20 minutes)

**Two main goals:**

1. Bring together some clinical development concepts when thinking about designing clinical trials that have the objective of determining the safety and efficacy (activity) of a novel medicine, highlighting some of the complexities and considering potential solutions
2. Consider which aspects of the study design and development programme are likely to benefit most from patient engagement

**The overall aim of this exercise is to demonstrate some of the real issues that might need to be addressed when considering the design of a pivotal clinical trial, including weighing up different options and thinking about how patients may provide added value in scientific advice / engagement with sponsors.**

**Case study brief:**

The clinical trial you will consider relates to an advanced cancer patient population, affecting mainly adults but also occurring in children. The cancer is rare and affects a total of 25,000 patients in the EU. Specific cancer promoting gene mutations have been identified and the cancer is increasingly being defined by rarer subgroups. The most characterised mutation results in overexpression of the BAD protein receptor, affecting 5% of those diagnosed with the cancer and a marker of poor prognosis.

There are 2 authorised medicines for treating the cancer and another medicine that is used very extensively ‘off label’ (unlicensed use). The disease is not well controlled and life expectancy is poor, with an average survival of 1 year. International treatment guidelines exist which state there is evidence of equal efficacy (anticancer activity) for the two authorised medicines.

The medicinal product is a novel tablet formulation called BADDER, that targets and inhibits the proliferative (growth) activity generated by the overexpression of BAD protein receptor. BADDER has had some early success in other similar cancers who carry the same BAD mutation. Treatment responses are usually observed after 6 months of therapy and the tablet must be given twice daily on an empty stomach. Tolerability can be an issue with this new drug and elderly patients in particular have reported severe fatigue, heart palpitations and debilitating diarrhoea.

* **Split into groups** for 3 rounds of brainstorming in small groups & feedback in plenary:

First round (20’): Work in your groups to consider how you might design the Pivotal study (e.g. number of study arms, duration, randomisation), patient selection (inclusion & exclusion criteria), dosing strategy, potential extrapolation of data, Comparator(s) licensed and off-label

Feedback discussion (20’): what type of study design did you select and why? What are your main inclusion exclusion criteria?

Second round (15’): Work in your groups to consider the study objectives and related primary and secondary endpoints, how and why did you select them

Feedback discussion (15’): What is your primary study objective? What endpoints are you interested in your study? How do you know that the chosen endpoints reflect what matters most to patients? How will you manage the safety and tolerability concerns?

Third round (10’): Work in your groups to consider Centre selection and sites. Monitoring of the patients during the study. Long term follow-up / additional studies/ use of real-world data post approval

Feedback discussion (10’)

* **Wrap up & Conclusions (10’):** Key elements to take away