

# EURORDIS MRD School 2025

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## Designing a patient-centric clinical development programme

Dan O'Connor, ABPI, UK

Virginie Hivert, EURORDIS, France

## 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**

## Overall aim of the exercise

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.

# Study brief 1 – the patients

Advanced cancer  
patient population



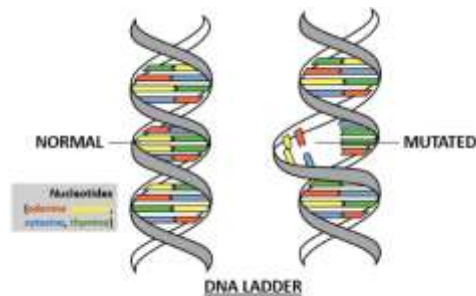
Prevalence of the  
disease

25,000 patients in  
the EU  
= 0.5 out of 10,000

Total pop. 500 millions

Rare

Cancer promoting  
gene mutations

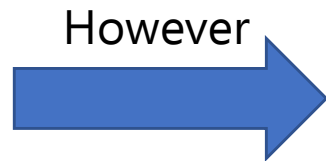
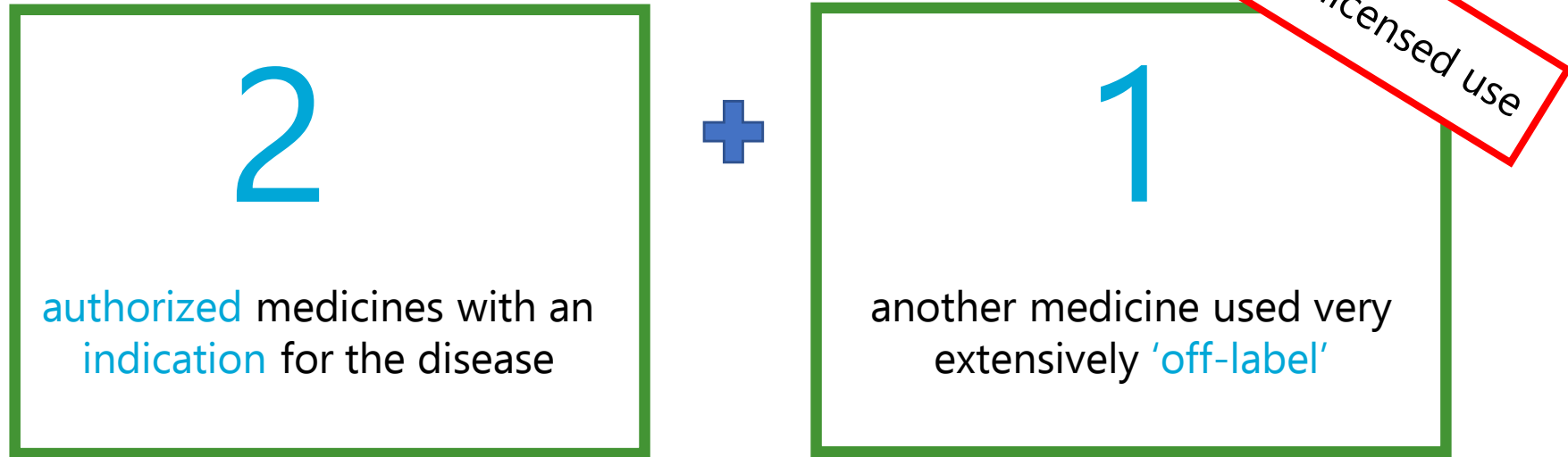


Overexpression of the  
BAD protein receptor



5% of the diagnosed patients  
/ poor prognosis

## Study brief 2a – therapy state-of-play



However

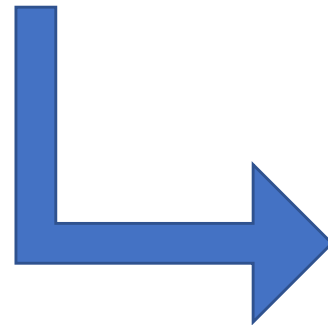
The disease is not  
well-controlled  
and life  
expectancy is  
poor

**Average  
survival  
1 year**

## Study brief 2b – therapy state-of-play

2

authorized medicines with an  
indication for the disease



Equal efficacy  
(anticancer activity)  
according to  
international treatment  
guidelines

# Study brief 3 – the new idea !

Medicinal product to  
be tested = **tablet**



Product name = **BADDER**

BADDER has been used  
**successfully** in similar  
cancers with the **same**  
**mutation**

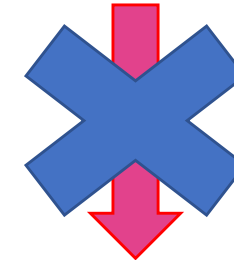


Mechanism  
of action

BADDER

BAD

BAD Rec



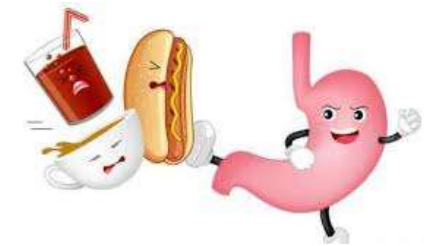
Proliferation  
of cells



# Study brief 4 - what we know about BADDER



6 Months



## Tolerability - side effects

Severe fatigue, heart palpitations and debilitating diarrhoea have been reported

In particular by elderly patients



# Discussions – 1<sup>st</sup> Round (20' + 20' feedback)

- Pivotal study design (number of arms, duration, randomisation),
  - Patient selection (inclusion & exclusion criteria),
  - Dosing strategy,
  - Potential extrapolation of data,
  - Comparator(s) licensed and off label
- 
- **What type of study design did you select and why?**
  - **What are your main inclusion exclusion criteria?**

## Discussions – 2nd Round (15' + 15' feedback)

- Study objectives and related primary and secondary endpoints
- **What is your primary study objective?**
- **What endpoints are you interested in your study?**
- **How do you know the chosen endpoints reflect what matters most to patients?**
- **How will you manage the safety and tolerability concerns?**

## Discussions – 3rd Round (10' + 10' feedback)

- Centre selection and sites
- Monitoring of the patients during the study
- Long-term follow-up / additional studies/ use of real-world data post approval

# Conclusions

- Where does patient engagement add value to the design of the study?
- Where patient engagement will be of most value?



# Thank you

<https://openacademy.eurordis.org/>