Laura Arenare, MSc

Clinical Trial Unit
National Cancer Institute of Naples
ITALY

ISTITUTO NAZIONALE TUMORI
IRCCS - Fondazione Pascale

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

New research approch
Patients Journey studies

ISTITUTO NAZIONALE TUMORI
IRCCS - Fondazione Pascale

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Background

New treatments and treatment combinations are becoming available for solid tumors

Trials performed to obtain registrations leave many questions unanswered, first of all on place-in-therapy and treatment sequences

Regulatory agencies are called to decide on the registration and reimbursement

However, sometimes, the available data are not sufficient to fully understand the value, the positioning and economic consequence of new drugs

Open access Communication

BMJ Open

The opportunity of patient-journey studies for academic clinical research in oncology

Francesco Perrone ,[1] Raimondo Di Liello ,[1] Piera Gargiulo,[1] Laura Arenare,[1] Lorenzo Guizzaro,[2,3] Paolo Chiodini ,[2] Ciro Gallo ,[2] Maria Carmela Piccirillo[1]

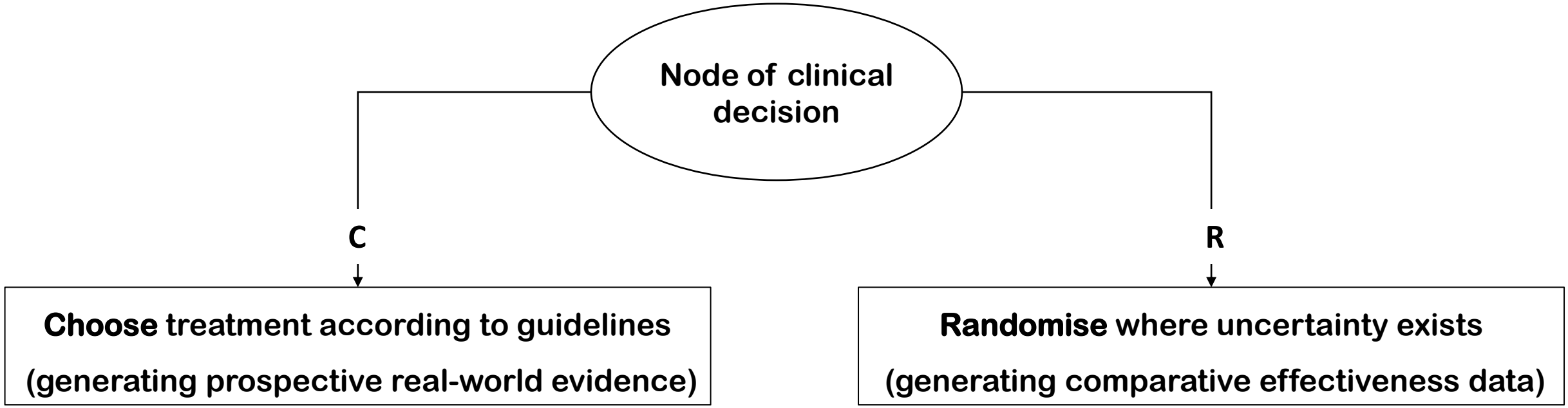
BMJ Open 2021

ISTITUTO NAZIONALE TUMORI
IRCCS - Fondazione Pascale

PJS

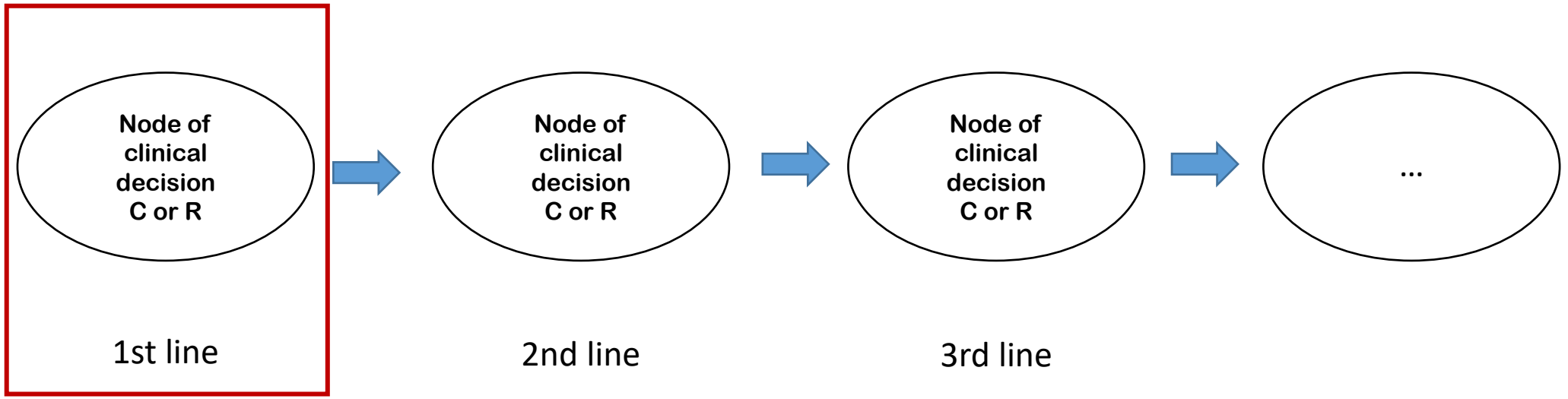
The idea is that patients are followed from the initial diagnosis across subsequent lines of treatment.

At each line of treatment…



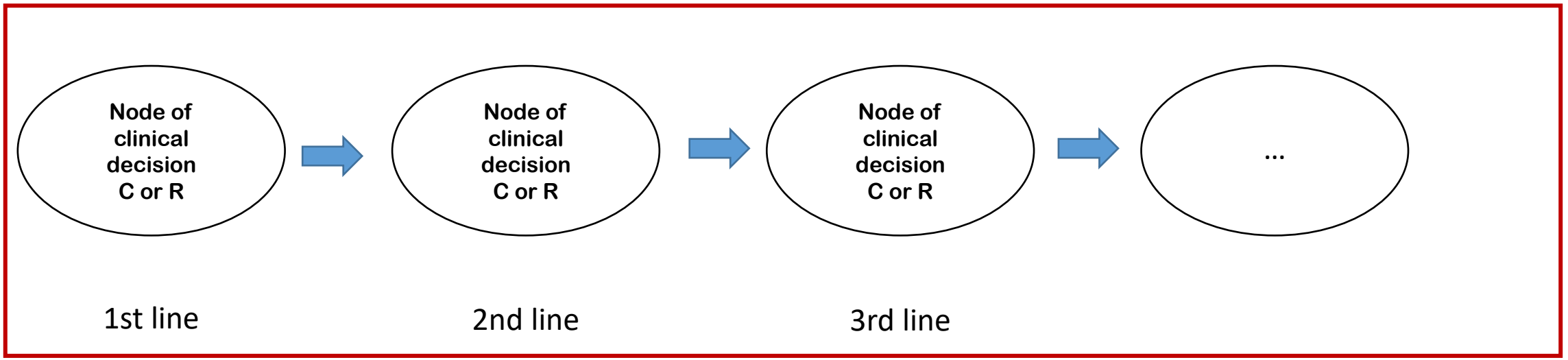
PJS

In this scenario, it is possible to answer specific questions within specific line, as is typically done in a clinical trial.



PJS

Moreover, it is possible to address questions regarding the effectiveness of therapeutic sequences.



The study of therapeutic sequences in a PJS can be conducted by randomizing the sequences or analyzing the choices made at each of previous clinical decision nodes.
The randomization allows us to use the standard methodology applied in clinical trial.
Outside randomization, the study of therapeutic sequences should be investigated using alternative methodologies as time-dependent approach.

Strengths of the PJS

- Adaptive Master Protocol
- Generates or increases knowledge on therapeutic sequence
- Improves understanding of the place-in-therapy for currently available drugs and those that will become available in future
- Evaluation of the entire therapeutic process, not just a segment of it
- Promotes integration between the real world and randomized data
- Creates the possibility of pharmacoeconomic analyses based on real data of drug efficacy and social context

Methodological challenges of the PJS

- Sample size estimation (adaptative design…)
- Multi-arm study (hierarchical approach…)
- Multiple comparisons (controlling alpha error…)
- Non proportionality of the hazard for comparisons of treatment sequences (time-dependent approach)
- The new evidence produced may generate new questions or lead to closure of arms where the treatment has not demonstrated any efficacy (interim-analyses…)

All these methodological solutions should be adapted to the PJS…

Thanks

l.arenare@istitutotumori.na.it

ISTITUTO NAZIONALE TUMORI
IRCCS - Fondazione Pascale

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH