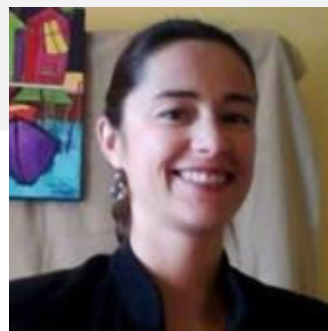# Real World Data in Oncology:
uses and new perspectives

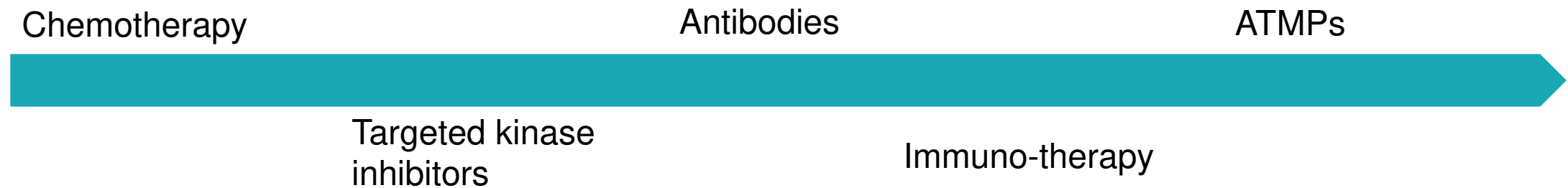**Louise BASCHET**   **Thomas FILLERON**   **Goulven THEZE**

Real World Data to support innovation in oncology

Reliable Real World Data in Oncology
contactRWD@unicancer.fr

Innovation increased survival by 2,7 years (1999-2016)

- **Innovation impact on patient's care**

Chemotherapy

Antibodies

ATMPs

Targeted kinase inhibitors

Immuno-therapy

1999-2016 increase of survival by 2,7 years.

Lichtenberg, F. R. (2023). The Relationship Between Pharmaceutical Innovation and Cancer Mortality in Spain, From 1999 to 2016. *Value in Health*, *26*(12), 1711-1720. link
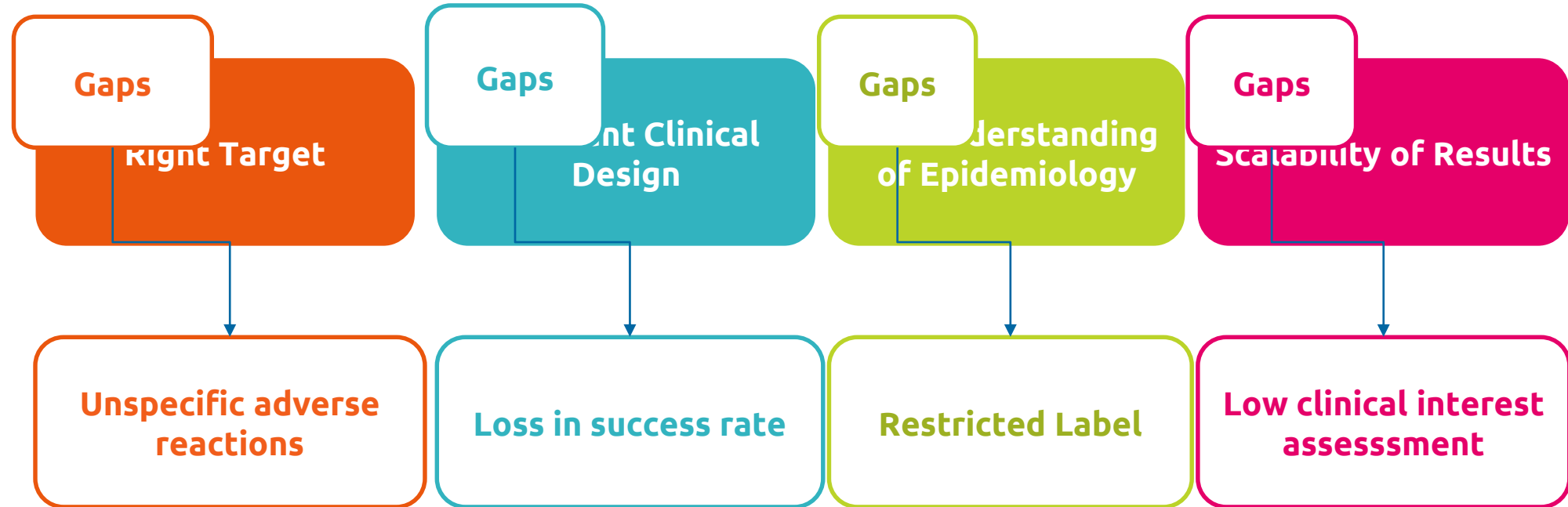
Challenges to be addressed in drug development
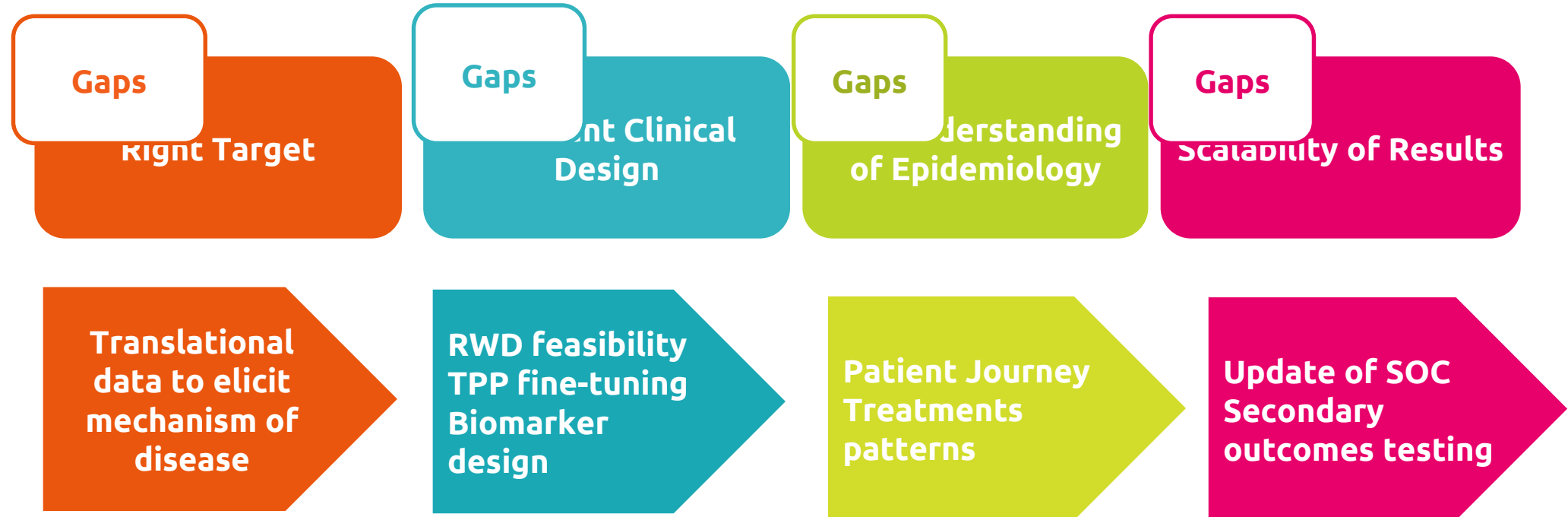
Right Target	Relevant Clinical Design	Full Understanding of Epidemiology	Scalability of Results

Risks of gaps in the drug development workstream

Gaps

Right Target

Gaps

...nt Clinical Design

Gaps

...derstanding of Epidemiology

Gaps

Scalability of Results

Unspecific adverse reactions

Loss in success rate

Restricted Label

Low clinical interest assesssment

How RWD is bridging these gaps

Gaps

Right Target

Gaps

...nt Clinical Design

Gaps

...derstanding of Epidemiology

Gaps

Scalability of Results

RWD Value

Translational data to elicit mechanism of disease

RWD feasibility TPP fine-tuning Biomarker design

Patient Journey Treatments patterns

Update of SOC Secondary outcomes testing

Lim, Z. F., & Ma, P. C. (2019). Emerging insights of tumor heterogeneity and drug resistance mechanisms in lung cancer targeted therapy. *Journal of hematology & oncology*, *12*(1), 134.link

Walker, I., & Newell, H. (2008). Do molecularly targeted agents in oncology have reduced attrition rates?. Nature reviews Drug discovery, 8(1), 15-16 link
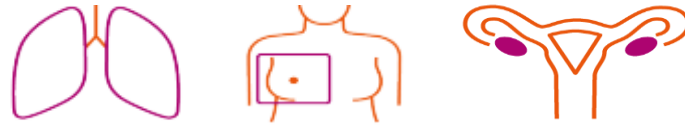
Bakker, E., Plueschke, K., Jonker, C. J., Kurz, X., Starokozhko, V., & Mol, P. G. (2023). Contribution of Real-World Evidence in European Medicines Agency's Regulatory Decision Making. *Clinical Pharmacology & Therapeutics*, *113*(1), 135-151. link

Campbell, U. B., Honig, N., & Gatto, N. M. (2023). SURF: AS creening Tool (for Sponsors) to Evaluate Whether U sing Real-World Data to Support an Effectiveness Claim in an FDA Application Has R egulatory F easibility. *Clinical Pharmacology & Therapeutics*. link

TPP : Target Product Profile
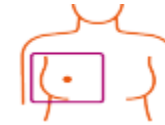**SOC :** Standard Of Care

Unicancer RWD programs

ESMÉ

canto
CANcer TOxicities

Mature programs with long term data

Retrospective Cohorts

Prospective Cohorts

Unicancer RWD programs

ESMÉ

canto
CANcer TOxicities

Mature programs with long term data

Retrospective Cohorts

Prospective Cohorts

New programs

ONCO DATA HUB

canto
CANcer TOxicities

INNOV
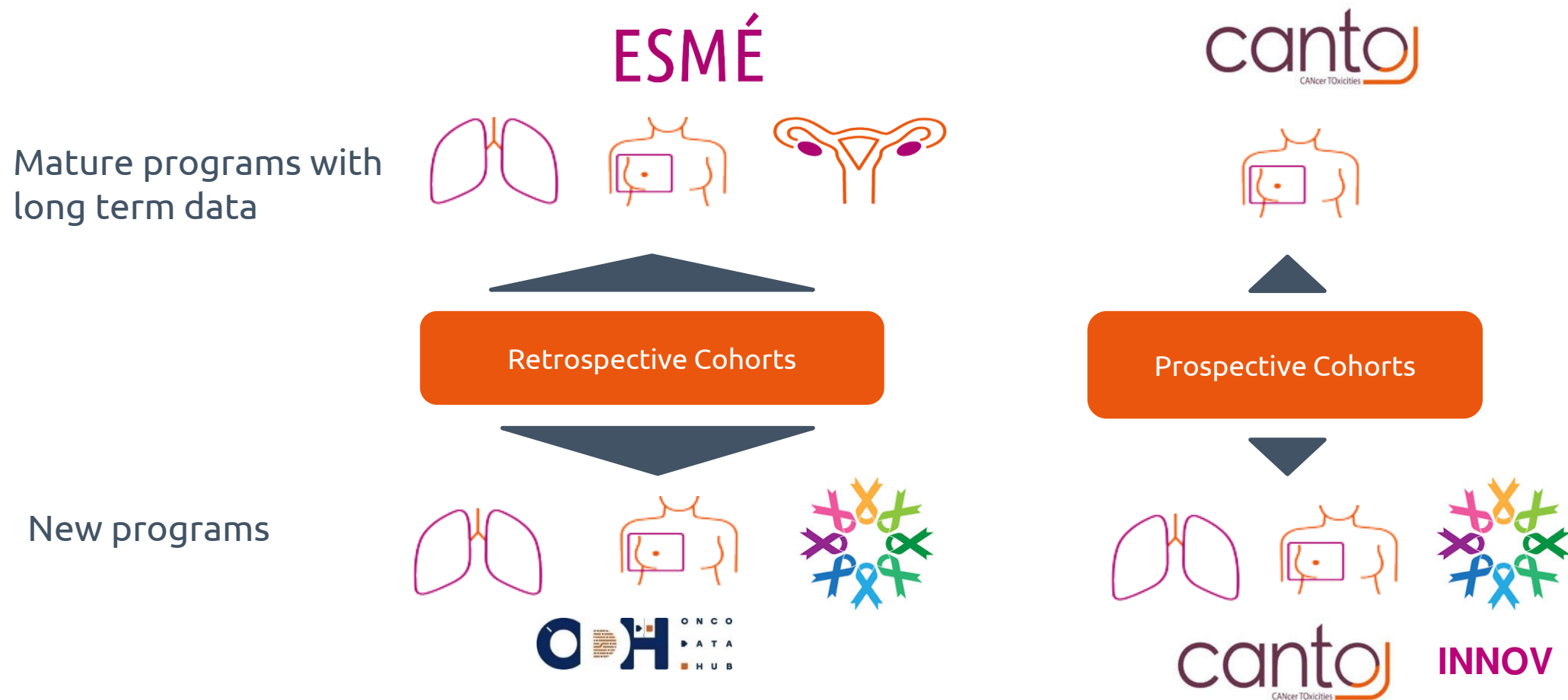
Harmonie Unicancer RWD linked to National Health Data

- **Integration of the 3 Unicancer programs with a linkage to the national health data records to reach completeness of patients' pathway**

ESMÉ

canto
CANcer TOxicities

ODH
ONCO
DATA
HUB

SNDS
Système national des données de santé

Harmonie **Data Warehouse**

Health dAta to foster Meeningfull OncologIc Evaluations

SNDS (French National Health Data Base) :
<u>Ambulatory care :</u> Demographics, Chronic diseases registration, Medical non medical encouters, Rx, Medical devices, Lab tests, costs
<u>Hospitalization :</u> Acute post acute care, Diagnoses, Procedures, costs
<u>Death certificates :</u> Date of death, Causes of death
  99% representativity, +20 years in depth

unicancer

Addressing scientific questions with RWD

ESMÉ
Detailed RWE

- Epidemiology
- Target population
- Sub groups of patients characteristics

- Therapeutic sequences evaluation
- Effectiveness
- HEOR

- RW Outcomes
- Synthetic control arms
- Target trial emulation

Accredited by French HTA for technology assessment process

HAS
HAUTE AUTORITÉ DE SANTÉ

canto
CANcer TOxicities

Biological & patient reported results RWE

-Epidemiolgy, risk factors
-Disease burden
-Biomarkers

-Toxicities and predictive factors
- Analyisis of specific sub groups
- Treatments impact on QoL

-Synthetic control arm
- AI for predictive factors

ODH ONCO DATA HUB
Treatments diffusion

- Patients/treatments profiles
- Treatment initiation by indications

-Treatments adoption /market share
- HEOR

- Treatment sequences description

Harmonie
Full course of care

- Epidemiplogy
- Patient management / ambulatory care

-Treatment patterns
- RW effectiveness
- HEOR

- AI for pronostic factors

unicancer

10

Example of publications ESMÉ

An Adjusted Treatment Comparison Comparing Amivantamab Versus Real-World Clinical Practice in Europe and the United States for Patients with Advanced Non-Small Cell Lung Cancer with Activating Epidermal Growth Factor Receptor Exon 20 Insertion Mutations

Christos Chouaid · Lise Bosquet · Nicolas Girard · Anna Kron · Matthias Scheffler · Frank Griesinger · Martin Sebastian · Jose Trigo · Santiago Viteri · Craig Knott · Bernardo Rodrigues · Nora Rahhali · Jedelyn Cabrieto · Joris Diels · Nolen J. Perualila · Claudio A. Schioppa · Jan Sermon · Raphael Toueg · Nicole Erdmann · Janka Mielke ·

Chouaid, C., Bosquet, L., Girard, N., Kron, A., Scheffler, M., Griesinger, F., ... & Wolf, J. (2023). An adjusted treatment comparison comparing amivantamab versus real-world clinical practice in Europe and the United States for patients with advanced non-small cell lung cancer with activating epidermal growth factor receptor exon 20 insertion mutations. *Advances in Therapy*, *40*(3), 1187-1203. link

cancers

Effectiveness of Nivolumab in Second-Line and Later in Patients with Advanced Non-Small Cell Lung Cancer in Real-Life Practice in France and Germany: Analysis of the ESME-AMLC and CRISP Cohorts

Christos Chouaid [1,*,†], Michael Thomas [2,3,†], Didier Debieuvre [4], Isabelle Durand-Zaleski [5], Stefan Zacharias [6], Lise Bosquet [7], Annika Groth [8], Annette Fleitz [9], Alan Calleja [10], Sonya Patel [10], Laure Lacoin [11], Melinda J. Daumont [12], John R. Penrod [13], Robert Carroll [14], Daniela Waldenberger [15], François-Emery Cotté [16], Clarisse Audigier-Valette [17,‡] and Frank Griesinger [18,‡]

Chouaid, C., Thomas, M., Debieuvre, D., Durand-Zaleski, I., Zacharias, S., Bosquet, L., ... & Griesinger, F. (2022). Effectiveness of Nivolumab in Second-Line and Later in Patients with Advanced Non-Small Cell Lung Cancer in Real-Life Practice in France and Germany: Analysis of the ESME-AMLC and CRISP Cohorts. *Cancers*, *14*(24), 6148. link

A WORKFLOW TO PERFORM MATCHING-ADJUSTED INDIRECT COMPARISONS WITH MULTIPLE IMPUTATION OF MISSING DATA ILLUSTRATED ON AGGREGATED SINGLE-ARM TRIALS AND THE ESME DATABASE

Cyril Esnault [1], Vanessa Barbet [2,3], Thomas Filleron [4], Gaëlle Chenuc [3], Maurice Pérol [5], Didier Debieuvre [6], Nicolas Girard [7], Xavier Quantin [8], Katia Thokagevistk [1], Gaëtane Simon [9], Louise Baschet [2,3]

Esnault, C., Barbet, V., Filleron, T., Chenuc, G., Pérol, M., Debieuvre, D., ... & Baschet, L. A workflow to perform matching-adjusted indirect comparisons with multiple imputation of missing data, illustrated on aggregated single-arm trials and the ESME database. link

OXFORD

Target trial emulation to assess real-world efficacy in the Epidemiological Strategy and Medical Economics metastatic breast cancer cohort

Alison Antoine, MSc,[1,2] David Pérol, MD,[1,*] Mathieu Robain, MD, PhD,[3] Suzette Delaloge, MD,[4] Christine Lasset, MD, PhD,[2,5] Youenn Drouet, PhD[2,5]

[1]Clinical Research and Biostatistics Department, Centre Léon Bérard, Lyon, France
[2]UMR CNRS 5558 LBBE, Claude Bernard Lyon 1 University, Villeurbanne, France
[3]Data Direction, UNICANCER, Paris, France
[4]Department of Cancer Medicine, Gustave Roussy, Villejuif, France
[5]Prevention & Public Health Department, Centre Léon Bérard, Lyon, France

Antoine, A., Pérol, D., Robain, M., Delaloge, S., Lasset, C., & Drouet, Y. (2023). Target trial emulation to assess real-world efficacy in the Epidemiological Strategy and Medical Economics metastatic breast cancer cohort. *JNCI: Journal of the National Cancer Institute*, djad092. link
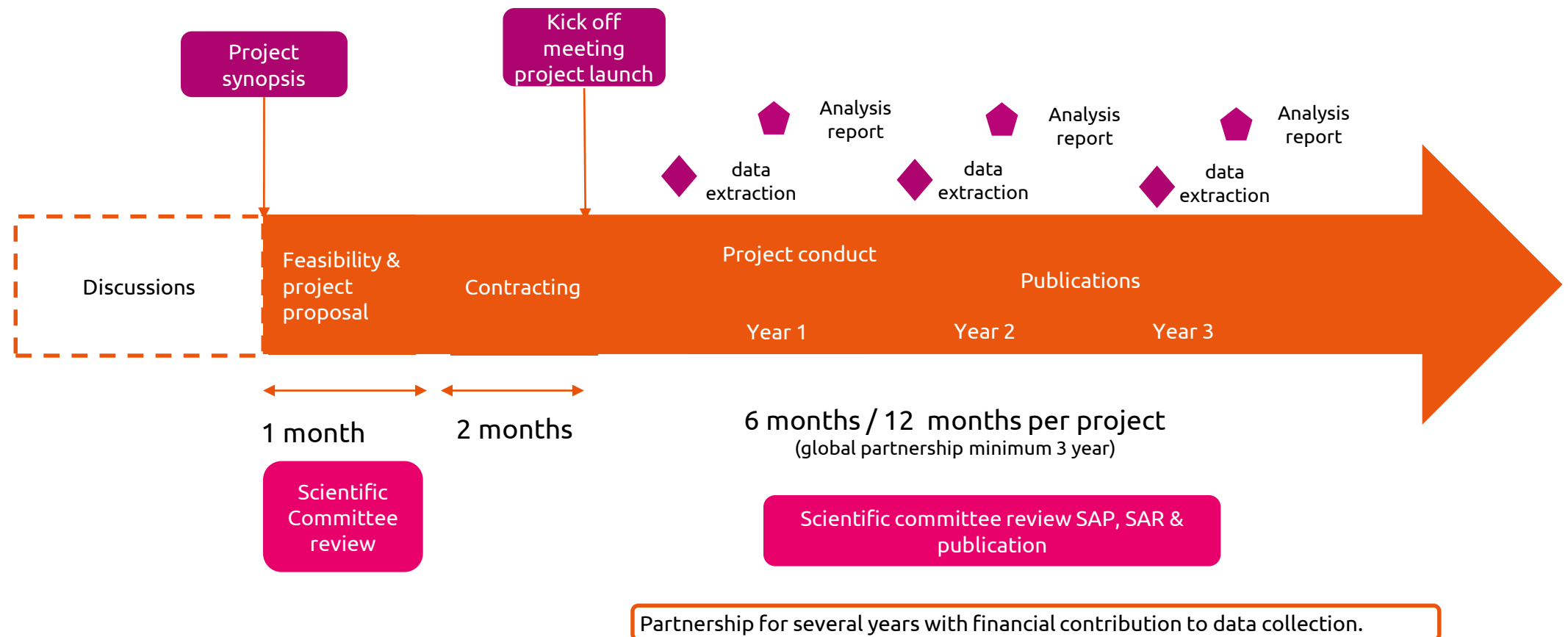
CrossMark

Cost-effectiveness of bevacizumab plus paclitaxel versus paclitaxel for the first-line treatment of HER2-negative metastatic breast cancer in specialist oncology centers in France

Audrey Petitjean[1], Jayne Smith-Palmer[2], William Valentine[2], Bertrand Tehard[3] and Stephané Roze[1]

Petitjean, A., Smith-Palmer, J., Valentine, W., Tehard, B., & Roze, S. (2019). Cost-effectiveness of bevacizumab plus paclitaxel versus paclitaxel for the first-line treatment of HER2-negative metastatic breast cancer in specialist oncology centers in France. *BMC cancer*, *19*(1), 1-11. link

unicancer

Project timelines

Project synopsis

Kick off meeting project launch

Analysis report

Analysis report

Analysis report

data extraction

data extraction

data extraction

Discussions

Feasibility & project proposal

Contracting

Project conduct

Publications

Year 1

Year 2

Year 3

1 month

2 months

6 months / 12 months per project
(global partnership minimum 3 year)

Scientific Committee review

Scientific committee review SAP, SAR & publication

Partnership for several years with financial contribution to data collection.

Major partnerships with healthcare companies

And active relations with health and data bodies for the sustainability and the expension of these programs

Contact

Unicancer
Direction Data & Partenariats
Okabé, 67 Av. de Fontainebleau
 94270 Le Kremlin-Bicêtre
Website :
https://recherche.unicancer.fr/en/health-data-research/

contactRWD@unicancer.fr