



The SMAC Club

The **SMAC club** (Statistics & Mathematics Applied to Cancer) brings together statisticians and mathematicians from the *Cancéropôle GSO* wishing to participate in a community of researchers interested in cancer research. The *Cancéropôle GSO* brings together about 500 scientific and medical research teams from the South West of France (GSO = Grand Sud Ouest = South West French Region) around collaborative projects. The *Cancéropôle GSO* runs a dynamic cancer research network and offers coordinated synergy between Institutions, Territories and Disciplines for the benefit of the patient.

Objective of the **SMAC Club** is to strengthen links between biostatistics, statistics and mathematics teams, as well as with teams who, in human and social sciences and epidemiology, use statistics. It aims both to help develop the skills of statistical teams, and to make these skills better known to “statistics users”. To do this, it notably organizes an annual conference on a theme chosen by the club. The themes that have been worked on by the SMAC club in previous years are very varied (cohorts, dynamic prediction models, quality of life, test simulation, imaging). The 2025 edition will focus on sample size issues in clinical research.

>> smac.canceropole-gso.org

SMAC Scientific committee

- **Carine Bellera** – Bordeaux Population Health Equipe EPICENE (Inserm U1219) et Inserm CIC1401 (Institut Bergonié, CLCC), Bordeaux
- **Thomas Filleron** – Biostatistics & Health Data Science, Institut Claudius Regaud IUCT-Oncopole, Toulouse
- **Kamel Gana** - Université de Bordeaux
- **Sophie Gourgou** - Unité de Biométrie, Institut du Cancer de Montpellier
- **David Jegou** - Institut de Recherche Pierre Fabre, Toulouse
- **Simone Mathoulin-Pélissier** – Bordeaux Population Health Equipe EPICENE (Inserm U1219) et ISPED (Université de Bordeaux) et Inserm CIC1401 (Institut Bergonié), Bordeaux
- **Caroline Mollevi** – Unité de Recherche Clinique & Epidémiologie, CHU de Montpellier, Montpellier
- **Virginie Rondeau** – INSERM U1219 (Biostatistics Team), ISPED, Université de Bordeaux, Bordeaux
- **Nicolas Savy** – Institut de Mathématique de Toulouse
- **Jean-Marie Boher** - Institut Paoli Calmettes, Marseille
- **Emmanuel Chamorey** - Centre Antoine Lacassagne, Nice
- **Roch Giorgi** - SESSTIM, Aix-Marseille Université

SMAC 2025 Local organization

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 Virginie Rondeau INSERM U1219, Bordeaux University

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Annual SMAC workshop
Statistics & Mathematics Applied to Cancer
January 23-24, 2025, Bordeaux, FR

**Sample Size Estimation in Clinical Research:
Recent statistical developments**

Abstract

When planning a clinical study, assessment of the required number of subjects to include is an essential step. Methods for estimating the sample size depend on the research question, the study design and the nature of the primary endpoint. Although there is a vast literature dealing with sample size estimation, a number of commonly used formulas remain suboptimal: sources of variability may be ignored, the underlying distributions of the endpoint may be misspecified, etc. As a result, sample size may be underestimated, leading to a lack of power and/or biased estimations of the treatment effect.

During this 1.5-day workshop, we will address statistical concepts for sample size estimation in clinical research, in the context of innovative trial designs and non-standard primary endpoints. Sample size for prediction models development will also be discussed, an issue specifically relevant with the ever-increasing use of IA tools in clinical research.

January 23, 2025	
8:45	Welcome / Coffee
9:15	Introduction (CB/VR)
9:30	[SESSION 1] Sample size: What for?
	Sample size determination: a key step when designing clinical trials
9:30	Eric Frison, CHU Bordeaux & Inserm CIC1401-EC, Bordeaux, France <i>10' talk + 5' discussion</i>
	Position of regulatory health authorities regarding new clinical trial design - 2024 F-CRIN Report
9:45	Louise Baschet, Horiانا, Bordeaux, France. <i>20' talk + 10' discussion</i>
10:15	Coffee break
10:45	[SESSION 2] Sample size & innovative trial endpoints
	Sample size, Generalized Pairwise Comparisons (GPC) and Net Treatment Benefit (NTB)
10:45	Marc Buyse, International Drug Development Institute, San Francisco, CA, USA; Hasselt University, Belgium. <i>20' talk + 10' discussion</i>
	The challenge of sample size determination in clinical trials with multiple co-primary endpoints
11:15	Jean-Marie Boher, Biostatistics and Methodology Unit, Institut Paoli-Calmettes, INSERM, IRD, SESSTIM, Aix Marseille Univ, Marseille, France <i>20' talk + 10' discussion</i>
	Sample size & recurrent events
11:45	Derek Dinart, CIC-EC 14.01, Institut Bergonié, Comprehensive Cancer Center & INSERM U1219, Bordeaux, France. <i>20' talk + 10' discussion</i>
12:15	Lunch & coffee
13:30	[SESSION 3] Sample size & innovative trial designs
	Sample size in mouse clinical trials
13:30	Hélène Savel, INSERM U1219, Bordeaux, France. <i>20' talk + 10' discussion</i>
	Innovative designs, extrapolation, simulation methods and evidence-tools for rare diseases
14:00	Sarah Zohar, INSERM INRIA UPC, Paris, France. <i>20' talk + 10' discussion</i>
	Sample size & innovative statistical designs to account for heterogeneity in cancer phase 2 trials
14:30	Bastien Cabarrou, Oncopôle Claudius Regaud, Toulouse, FR. <i>20' talk + 10' discussion</i>
15:00	Coffee break
15:30	[SESSION 4] Sample size & cluster randomized trials
	Cluster randomized trials: overview and general sample size considerations
15:30	Agnès Caille, Tours University & INSERM U1246, FR. <i>20' talk + 10' discussion</i>
	Sample size considerations for stepped-wedge and cluster crossover designs
16:00	Laurent Billot, The George Institute for Global Health, UNSW Sydney & Bordeaux University. <i>20' talk + 10' discussion</i>
16:30	End of the session
20:00	Diner in town (speakers & scientific committee)

January 24, 2025

8:30 *Coffee*

9:00 [SESSION 5] **Sample size, prediction & AI**

Sample size for the development & validation of a clinical prediction model

9:00 Marteen Van Smeden, UMC Utrecht, the Netherlands

20' talk + 10' discussion

Survival prediction from high-dimensional biomarker data in clinical trials

9:30 Stefan Michiels, Institut Gustave Roussy & INSERM U1018, Paris, FR.

20' talk + 10' discussion

TRIPOD + AI 2024 statement: enhancing the transparency of clinical prediction models that use regression or machine learning methods

10:00 Karel Moons, UMC Utrecht University, Utrecht, the Netherlands

20' talk + 10' discussion

10:30 *Coffee break*

11:00 [SESSION 6] **AI & clinical applications : *next-generation* methodological challenges**

Challenges and limits of AI for routine use in medical imaging

Olivier Humbert, Interdisciplinary Institute for Artificial Intelligence, 3IA Université Côte d'Azur, Nice, France.

20'-30' talk

11:00 **Bridging the gap between clinical needs, AI opportunities & methodological challenges**

Rodolphe Thiébaud, Bordeaux University & INSERM U1219, Bordeaux, France.

20'-30' talk

Round table

12:00 Speakers of the day + Virginie & Carine.

30'

12:30 *End of the seminar & lunch*

