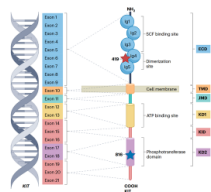


# Effectiveness and tolerability of avapritinib in indolent systemic mastocytosis : first results from the French nationwide real-world AIMS study



Clément Gourguechon, Cristina Livideanu, Maël Heiblig, Edwige Le Mouel, Stéphane Barete, Yannick Chantran, Ludovic Lhermitte, Oriane Debeaupuis, Hassiba Bouktit, Olivier Hermine, Julien Rossignol and CEREMAST investigators

## Systemic Mastocytosis



- Heterogeneous rare disease** (prevalence 1/3500-4000, incidence 1,21-1,56/100 000h/yr<sup>1</sup>)
- Myeloproliferative disorder** subcategory (WHO 2022)
- Abnormal tissue accumulation** of clonal mast cell
  - Immunophenotypic (CD25, CD2, CD30)
  - Morphology : spindle shape, hypergranular cytoplasm, atypic nuclei
  - Molecular : c-KIT D816V mutation
- Clinical Heterogeneity**
- 90 % are non advanced form**
  - Indolent Systemic Mastocytosis (ISM) is the most prevalent subtype
  - Anaphylaxis and severe osteoporosis can be inaugural
  - Altered QoL in most of patients related to mast cell activation symptoms
- Avapritinib**
  - First selective KIT inhibitor approved for ISM with moderate to severe symptoms despite best supportive cares
  - Demonstrate clinical efficacy on symptoms and biomarkers with good tolerability

<sup>1</sup> \*Estimation: Jorgensen Eur J Epidemiol 2025 Bergström Acta Oncologica 2024 Schuler JACI 2021. Kibgaard Int J Women Dermatol 2020. Cohen Bjh 2014. Van Doormaal JACI 2013; Khouri ;

Akin et al, Nat Rev Dis Prim. 2025  
Gotlib et al, NEJM Evidence. 2023

## AIMS study : a real world experience of avapritinib use in ISM

A Multicenter Nationwide Real-World Study Design



**Primary endpoints :** Quality of life Mc-QoL

Activity : UP self evaluation variation – TSS Total Symptoms Score (ISM-SAF)

Safety : Anaphylactic and bone events

Biology : Tryptase –KIT VAF (Variant allelic Frequency) - CTX (Beta Crosslaps)

DXA : bone mineral density assessment

Treatments : Mediators ciblign and symptomatic treatments

**Avapritinib :**

**Safety**

Discontinuation, dose reduction or increase

## AIMS study : a French nationwide multicentric study



n= 162



April 2024 to April 2026



CEREMAST NETWORK = 21 centers



Indolent systemic mastocytosis according WHO 2016



Debilitating symptoms despite best supportive symptomatic cares

## AIMS study : a real life population



Women : 63,6 %



Anaphylaxis : 19,8 %



Median age : 54,4 years



Osteopenia : 26,5 %  
Osteoporosis : 22 %  
Bone Fracture : 22 %



Skin involvement : 89 %



Mutation KITD816V : 97 %  
VAF : 0,89 %



Previous Omalizumab treatment = 1/4

Tryptase : 47,8 µg/l

## AIMS study : first 6 month results

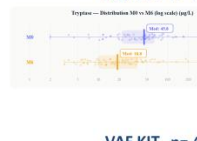
MC-QoL n=90



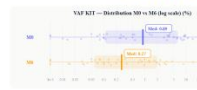
ISM-SAF n=78



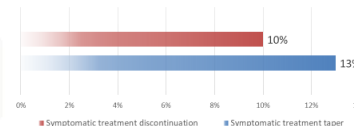
Tryptase n= 70



VAF KIT n= 45

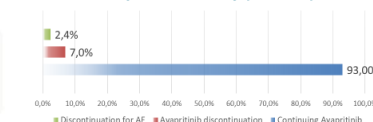


## Symptomatic treatment at 6 month n= 90



23 % discontinued or tapered at least one symptomatic treatment at M6

## Avapritinib Safety (n = 162)



Discontinuation n= 12 (2,6 month) For no severe AE (n=4) : oedema, diffuse pain, asthenia, digestive troubles