NEW THERAPEUTIC APPROACH FOR DIFFUSE MIDLINE GLIOMA: 3-YEAR EVALUATION OF ACCESS TO ONC201 IN FRANCE AND EUROPE

Lucas Denis[1*] ; Vignes Marina[1*] ; Daniela Di Carlo[2,3]; Jacques Grill[2]; Valérie Salomon[4]; André Rieutord[1] ; Maxime Annereau[1]

[*]These authors contributed equally to this job
[1] Department of Clinical Pharmacy, Gustave Roussy, 114 rue Édouard Vaillant, Villejuif
[2] Department of Pediatric and Adolescent Oncology
[3] University of Padova, Hematology Oncology division-department of Women's and children's health, Padova, Italy
[4]ANSM, National Agency for the Safety of Medicines and Health Product, Saint-Denis, France

3PC-036

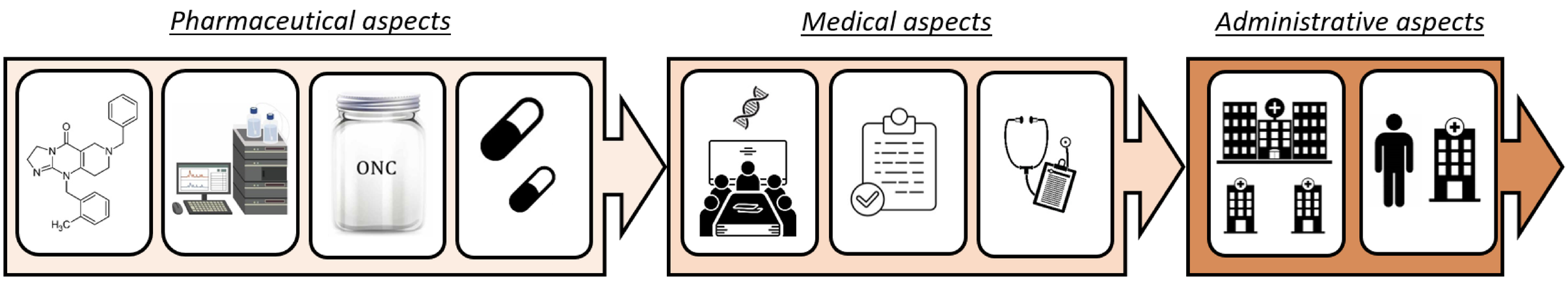
Background and Importance

H3K27M-altered diffuse midline gliomas have a poor prognosis in adults and children (**less than one year after the diagnosis**), with current treatment strategies relying on radiation therapy that merely slows disease progression. Recently, ONC201 (dordaviprone), a drug developed by Chimerix®, demonstrated promising results in a clinical trial conducted in the U.S. only. To address the lack of availability in France, the French Medicines Agency (**ANSM**), following recommendations from pediatric oncology and neuro-oncology experts, **initiated a compassionate use program led by Gustave Roussy**. This program allows French and European patients access to ONC201 through a compounding mechanism and the S2 reimbursement process.

Aim and Objectives

Ensure and centralize ONC201 safe access to patients in France and abroad.

Material and Methods



Pharmaceutical aspects

- *Qualification of raw material ONC201*
  - *Pharmaceutical-grade quality*
- *Capsules at 25 and 100 mg and oral solution*
  - *Stability evaluated by HPLC*

Medical aspects

- *Medical indication for ONC201 determined by multidisciplinary meetings*
  - *Consent to SACHA study*
  - *ONC201 prescription*

Administrative aspects

- *Inter-institutional agreement*
- *Out patient delivery by hospital pharmacy*

Results

Production of :

> 100 batches of capsules

20 batches of oral solution

With a **one-year stability**

Treatment of :

234 patients from 52 centres and **13 countries**

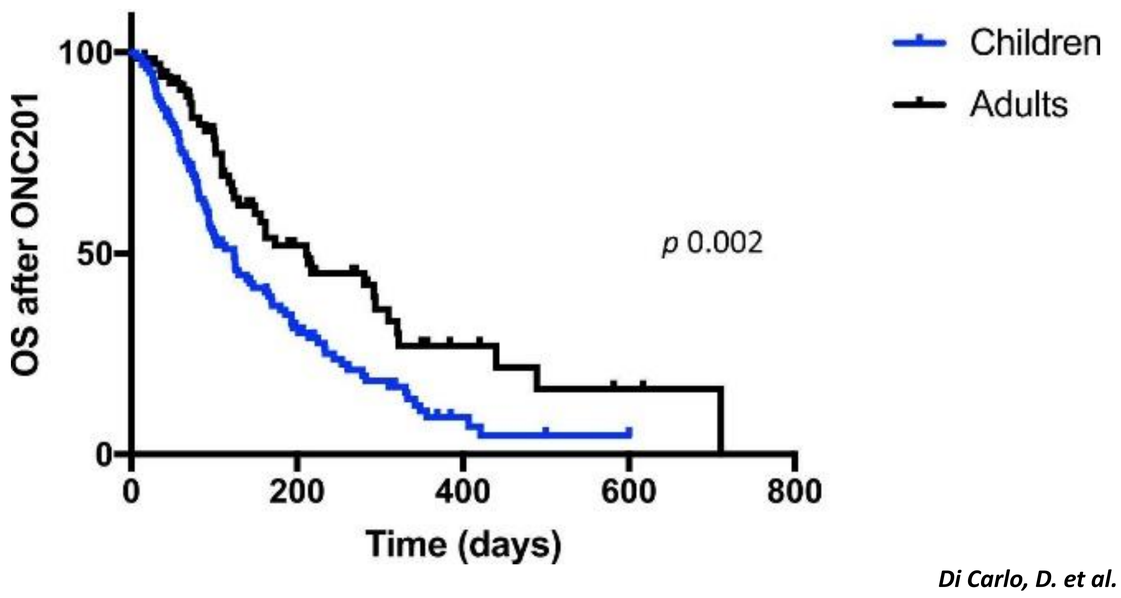
No severe adverse effects were reported

Median of **five** treatment **cycles**

10% of patients > 10 cycles

7 patients (**3%**) with a **prolonged response** > 18 months

4-month OS of 57.5%



Di Carlo, D. et al.
European Journal of Cancer, Feb. 2025 Volume 216

Conclusion and Relevance

This study highlights the clinical potential of ONC201, with encouraging outcomes compared to gold-standard radiation therapy. This **collaboration between patients associations, ANSM, clinicians and pharmacists** highlights the importance of hospital pharmacy for formulation development and manufacturing of safe medicines **to bridge the gap of unmet clinical needs** for rare disease in cancer treatment.

Lucas.denis@gustaveroussy.fr