

# BALANCING CLINICAL BENEFITS AND COST SAVINGS: COMPASSIONATE DRUG USE AT AN ITALIAN UNIVERSITY HOSPITAL – EVIDENCE AND INSIGHTS

Mezza M (1), Iadicicco G (2), Miscio MA (2), Brunoro R (2,3), Mengato D (2), Gregori D (1), Venturini F (2,3)

1) University of Padua, Biostatistics, Epidemiology, and Public Health Unit (UBEP), Padua, Italy – 2) University Hospital of Padua, Hospital Pharmacy, Padua, Italy – 3) University Hospital of Padua, Clinical Research Unit, Padua, Italy.

## BACKGROUND AND IMPORTANCE

In this study, a **retrospective analysis** of approved **compassionate uses of drugs** (CUs, Fig.1) at the Azienda Ospedale-Università di Padova (AOUP) between 2022 and the first half of 2023 was conducted, with a focus on the **economic-clinical implications**.

- ✓ Absence of feasible therapeutic alternatives;
- ✓ Positive evaluation by the Territorial Ethics Committee (TEC);
- ✓ Willingness of the manufacturer to supply the drug free of charge.



## AIM AND OBJECTIVES

The purpose is to emphasize the importance of CUs in a University Hospital, to ensure patients have access to **potentially life-saving therapies** without burdening National Health Care System's **economic budget**.

## MATERIALS AND METHODS

A **monitoring activity** was implemented by the AOUP Clinical Research Unit through creation of **follow-up forms** submitted to corporate Operational Units (OU) physicians in order to evaluate the **clinical and economic impact** of CUs (Fig.2).

### follow-up forms



### Clinical impact monitoring activity



- N° of patients;
- Clinical outcomes;
- Treatment duration.

### Economic impact monitoring activity



- Ex-factory price;
- Length of therapy;
- Posology and dosage.

COST-THERAPY

Fig.2

## RESULTS

- In the time frame considered, at AOUP, the CU regimen was approved for **84 patients** mainly afferent to the **hematologic field** (Fig.3).
- In 79 undertook the treatment, with, in **81%** of cases, **partial or complete improvement** of the clinical picture, or stability of disease, in the case of degenerative diseases (Fig.4).

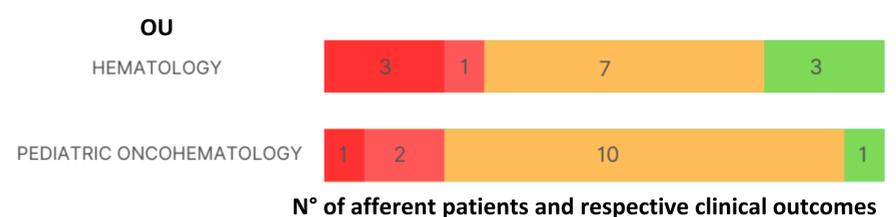


Fig.3

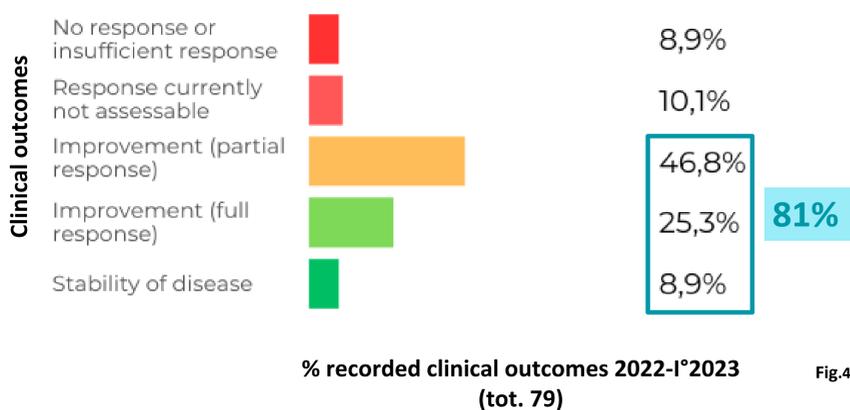


Fig.4

- The **avoided costs** amounted to **€7,130,668**, **62%** of which came from the compassionate use of the drug **burosumab** in patients with X-linked hypophosphatemic osteomalacia (Fig.5).

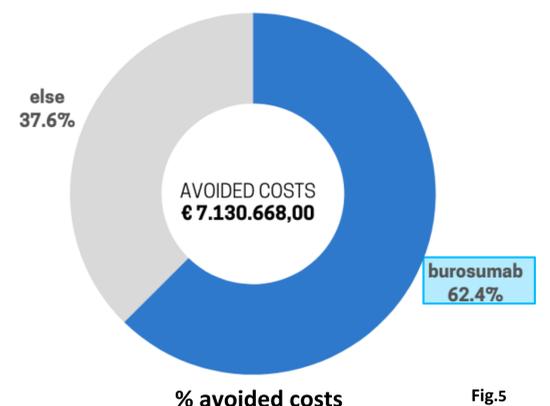


Fig.5

## CONCLUSIONS AND RELEVANCE

- Collaboration between **university hospitals**, **pharmaceutical companies** and **regulatory authorities** is essential to optimize compassionate drug use programs ensuring equitable access to potentially **life-saving treatments** and at the same time facilitating the gathering of valuable **clinical data**.
- **Cost savings** generated from this approach can be reinvested to **expand** the national healthcare system.

ABSTRACT NUMBER:

1ISG-012



## REFERENCES

D. Pilunni et. al, Eur Rev Med Pharmacol Sci 2021, 25,20, 6365.