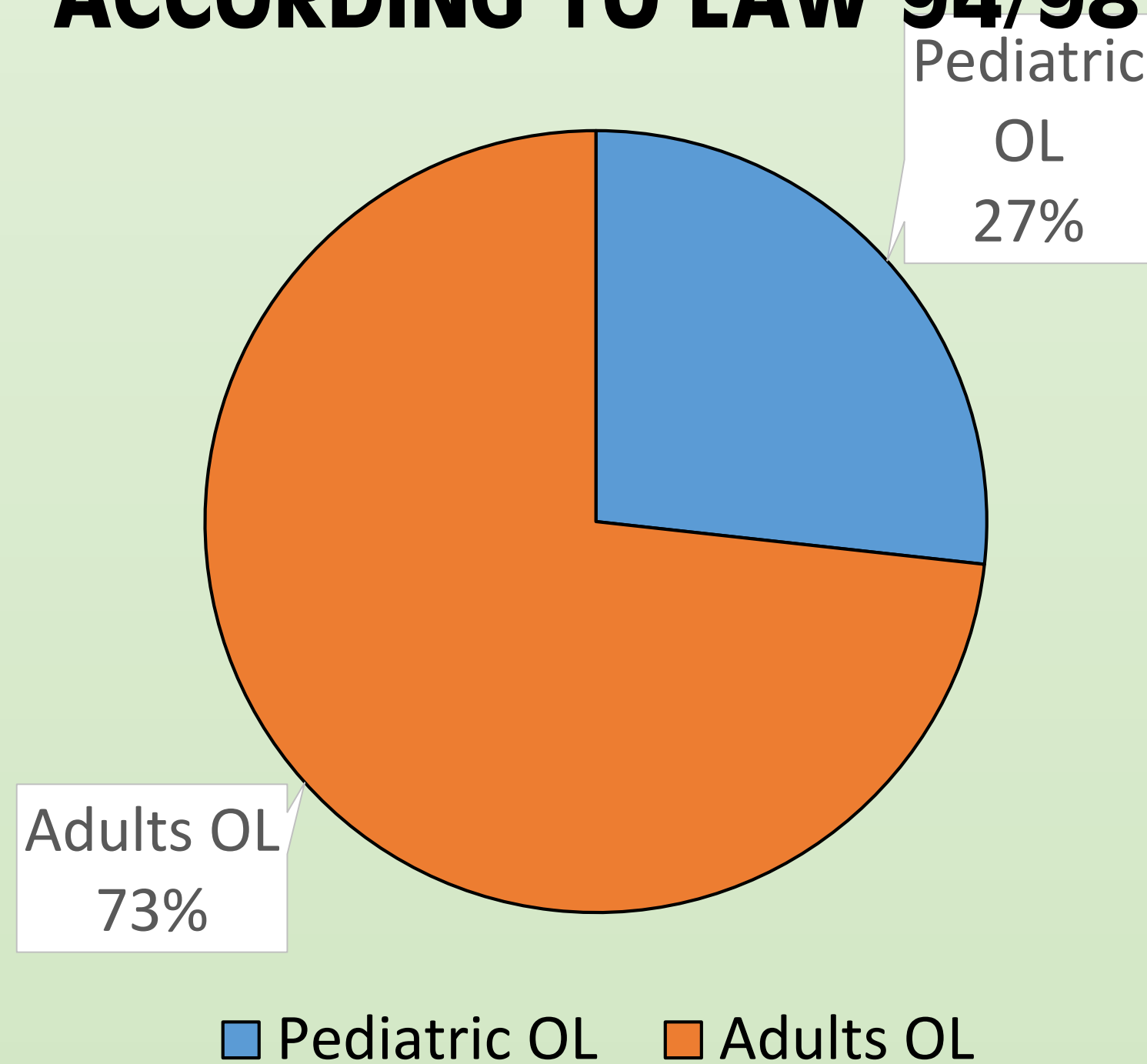


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Background and importance: Data concerning drugs' dose, efficacy and safety in pediatric are very limited and this gap of knowledge induces the off-label (OL) drug use. A study (1) showed that 60% of pediatric prescriptions were OL and the main OL drug classes were antibacterial, anti-asthmatic, analgesics. Over the last 30 years the Italian Drug Agency (AIFA) have approved laws to ensure an appropriate use of OL medications such as Law 648/96, Law 94/98 (it guarantees the OL drug use but it isn't supported by the National Health Institute (NHI)'s fundings), Law 326/03 (it ensures the NHI's fundings for the use of orphan and life-saving drugs before market authorization) and Law 7/9/2017.

Aim and objectives: The aim of this work is to evaluate the pediatric OL drug use and safety in an Italian hospital over the last 2 years according to Law 94/98 and Law 326/03.

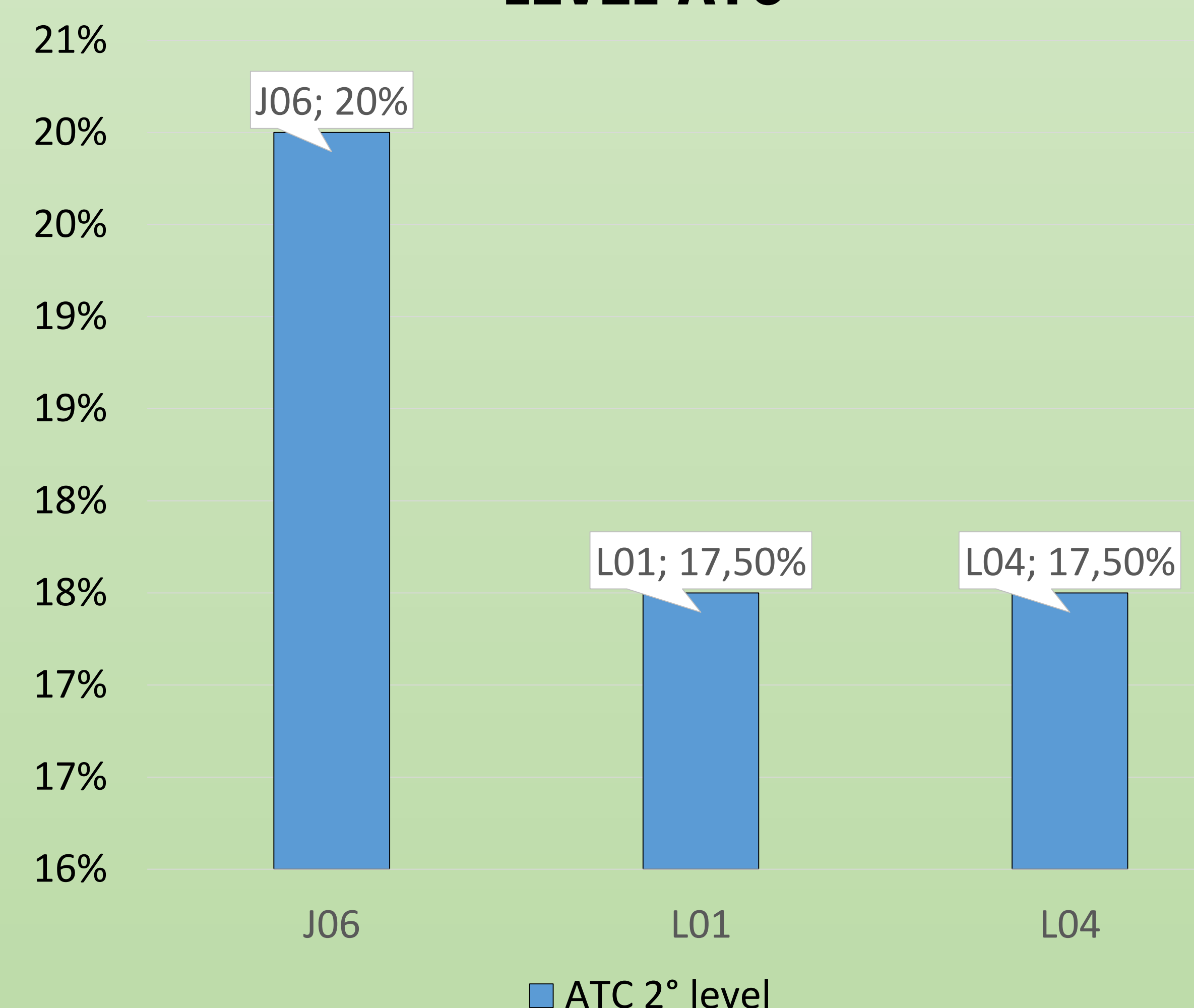
OFF LABEL PRESCRIPTIONS ACCORDING TO LAW 94/98



Materials and methods: We analyzed OL prescriptions evaluated by the Hospital OL Committee (HOLC) (composed by a Hospital Pharmacist, Pharmacologist, Clinician) from January 2021 to December 2022. We calculated how many pediatric patients were involved, which OL drug was the most prescribed and for what type of disease (if rare disease according to the Italian rare disease database *Malattierare.gov*), how many patients presented an Adverse Drug Reaction (ADR). We also considered OL all the Intravenous Immunoglobulins (Iglv) that weren't prescribed according to our regional *Operative Procedure for the appropriate use of Iglv*.

Results: From January 2021 to December 2022 the HOLC evaluated 258 OL prescriptions according to the Law 94/98 and 69 (27%) administered to 49 pediatric patients (2 patients received 2 OL drugs). 25 different OL drugs were used to treat 33 conditions (20 rare diseases); 7 drugs (28%) didn't have the pediatric license. The most prescribed OL drug (2nd level ATC) was the J06-Immune Serum and Immunoglobulins (20%) represented by Iglv to treat conditions such as Idiopathic Dermatomyositis, Giant cell Hepatitis with Autoimmune Hemolytic Anemia, Chronic Polyradiculoneuritis (with or without anti-MOG antibodies), Autoimmune Encephalitis, Rasmussen Syndrome, Opsoclon Myoclon Syndrome followed by the L01-Cytostatic (17,5%) represented by Bevacizumab to treat Glioma and L04-Immunosuppressant (17,5%) represented by Adalimumab to treat Bechet Syndrome and Systemic Vasculitis. In the same period 6 patients received OL drugs according to the Law 326/03 and 4 (67%) were pediatric. 3 OL drugs were used to treat 2 rare conditions: 2 patients received the combination therapy Ivacaftor/Tezacaftor/Elexacaftor + Ivacaftor to treat Cystic Fibrosis and 2 Fenfluramine to treat Dravet Syndrome. 4 ADRs referred to 4 OL therapies were reported in 4 pediatric patients induced by Ponatinib, Iglv, Arsenic-Trioxide, Rituximab.

OFF LABEL DRUGS BASED ON 2nd LEVEL ATC



Conclusion and relevance: The paediatric OL drug use in a common practice and over the last 30 years several strategies were adopted to guarantee an early and safe access. For example in our hospital, since 2007, all drugs included in the Hospital Therapeutic Formulary can be prescribed (without the HOLC's evaluation) if they are on label for indication but off label for age and/or dosage and/or frequency.

References: (1) C. Pandolfini et al, Off-label use of drugs in Italy: a prospective, observational and multicentre study, Acta Paediatr. 2002; 91(3):339-47

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