Orphan Drugs Availability in Israel – A Base for Policy Development

HPR153

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INTRODUCTION

Orphan drugs (ODs) represented 0.06% of total prescriptions in Germany in 2020, but 11.6% of the total expenditure on drugs[1]. The expense proportion on ODs in the US grew from 2% in 1992 to 11% in 2019[2]. Global expenses are expected to reach 20% in 2028[3]. There is no definition for an orphan drug, nor is there a specifically defined registration, pricing, or reimbursement process in the Israeli law [4].

OBJECTIVE

To map and analyze current global and Israeli approval of orphan drugs (ODs), identify key trends, and assess potential impact on the Israeli healthcare system, which currently lacks a formal definition and policy regarding orphan drugs

METHOD

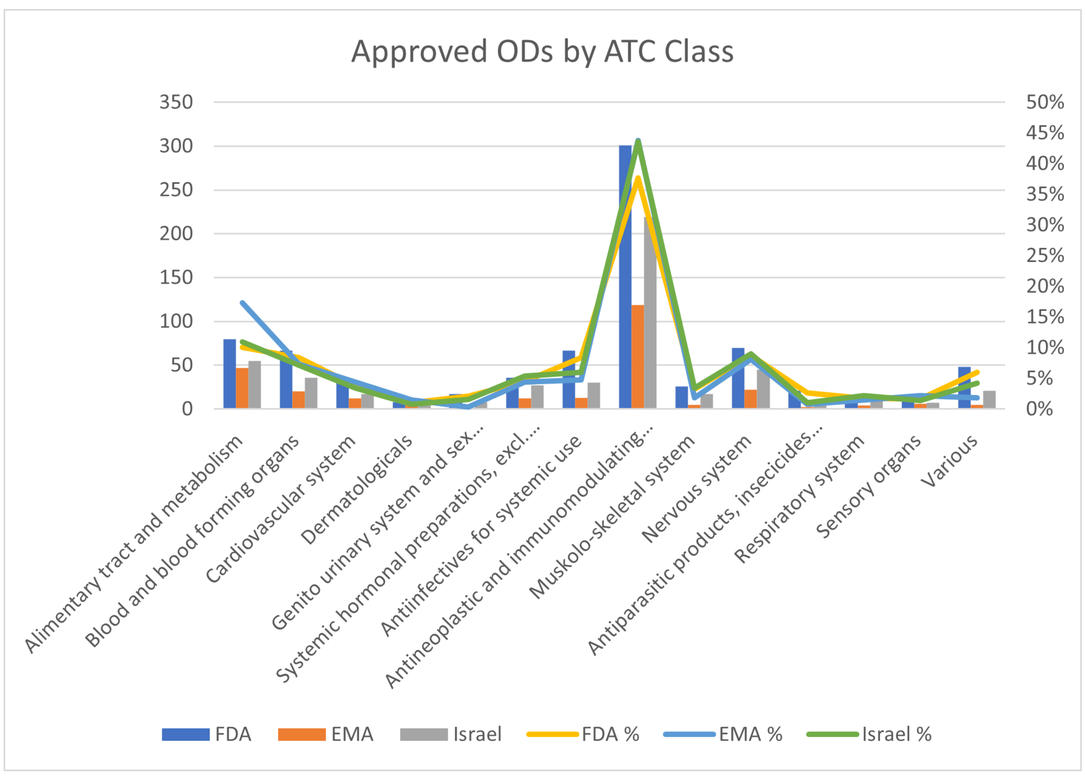
- **A comparative analysis** was conducted of all approved ODs by the FDA and EMA from January 2000 to December 2024, and all drugs approved in Israel (IL) up to February 2025.
- **Identification and matching** of drugs across the lists were performed using both trade and generic names, supplemented by manual verification when necessary.
- **Pharmacotherapeutic groups** were derived from the received EMA file; in cases of missing information, ATC Level II was added. Drugs were categorized according to ATC I.

RESULTS

During the study period, 789 ODs were approved by the FDA, 272 by the EMA, and 502 in Israel (matching either FDA or EMA). There were 243 ODs approved by both the FDA and the EMA, 191 (79%) of which are also approved in Israel. Additionally, 361 drugs are approved only by the FDA as ODs, and 185 are approved as ODs by the FDA and as regular drugs by the EMA.

Mapping of Orphan Drugs Registered in Israel

	FDA / EMA	Approved in Israel
OD-approved by FDA only	361	153 (42%)
OD-approved in both EMA and FDA	243	191 (79%)
Approved as OD by FDA only, EMA regular Approval	185	143 (77%)
OD-approved in EMA, not approved by FDA	20	9 (45%)
Approved as OD by EMA only, FDA regular Approval	9	6 (67%)
Total	818	502 (61%)



The predominant therapeutic area among approved ODs by FDA, EMA, and IL, respectively, is Antineoplastic and immunomodulating agents (38%, 44%, 44%), followed by Alimentary tract and metabolism (10%, 17%, 11%) and Nervous system (9%,8%, 9%).

CONCLUSIONS

Analysis of OD registration indicates that approximately 60% of drugs approved by either FDA or EMA are registered in Israel, with a trend toward antineoplastic agents and drugs approved by both the FDA and EMA, either as ODs in both or OD by the FDA and regular approval by EMA.

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