Rare Diseases and Orphan Drug Availability in Malta

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AIMS

Over 7000 rare diseases (RD) affect around 60 million patients in the European Union and the United States (US).

'RD-action' is a European Health Programme funded Joint Action which provides information and data on RDs.

The aims of the study were to describe orphan drug (OD) availability and the health needs of the Maltese RD population.

METHODS

Themes to assess RD management in Malta were adapted from the RD-action plan and were identified through literature concerning RDs in EU countries. The themes were used to formulate interview questions prior to meeting policy makers in the Department of Health Information and Research Malta to obtain information on availability to medication and treatment. Available RD registries were analysed and the 10 most common RDs in Malta identified.

RESULTS

Malta lacks specific RD policies, RD plans, clinical practice guidelines on RDs and an RD information centre (Table1). Four RD registers are active in Malta: Cancer Register, Congenital anomaly register, Malta RD register and Treated abroad RD register. Eight out of the 10 most reported RDs are cancers.

Out of the 10 most common RDs, only multiple myeloma has an approved OD which is bortezomib.

RD PLAN	Not specific to Malta
CENTRE OF EXPERTISE ON RDs	No
RD REGISTERS	Four registers currently active
SCREENING AT BIRTH	No legislative mandate
GENETIC DATABASE	Malta BioBank (launched in 2017)
CLINICAL PRACTICE GUIDELINES ON RDs	No
RD EDUCATION	Annual RD colloquium
ORPHANET TEAM	No
RD PATIENT HELPLINES	No
RD INFORMATION CENTRE	No
RESEARCH FUNDS DEDICATED FOR RDs	No
OD POLICY	No
NGOs SUPPORTING RDs	Yes (Marigold foundation)

Table 1: Summary of RDs and OD findings in Malta

CONCLUSION

There is a need for policy makers to focus on establishing an RD plan and increasing access and availability of ODs.