

Towards Shaping Rare Diseases and Orphan Drugs Health Ecosystem in Saudi Arabia



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INTRODUCTION

- Globally, the management of rare diseases (RDs) and access to orphan drugs (ODs) remain challenging, largely due to the absence of national strategies and defined access pathways in many countries.
- Although some countries have implemented policies and legislation concerning RDs and ODs, these are not universally applicable.
- The provision of RD care and access to orphan drugs (ODs) in Saudi Arabia remains challenging, despite substantial financial resources. Obstacles such as infrastructural limitations, workforce specialization gaps, and geographical disparities in accessing centers of excellence hinder the development of an equitable care continuum.
- The absence of a structured national strategy for RDs in Saudi Arabia contributes to fragmented policy implementation, delays in drug approvals, and inconsistencies in reimbursement policies.
- To mitigate these challenges, the Saudi health system is prioritizing collaborative governance and collective actions to bridge equity gaps in care.

AIM

- To analyze RDs landscape in Saudi Arabia and develop actionable strategy to improve RDs care provision and ODs access.

METHODS

- We convened a one-day, multi-stakeholder policy workshop in Riyadh, Saudi Arabia, in collaboration with the Saudi Health Council.
- Participants included official representatives from the public, semi-governmental, and private sectors, encompassing policymakers, regulators, providers, healthcare professionals, payers, academia, patient advocacy groups, and the pharmaceutical industry and insurance companies.
- Through presentations and plenary discussions, participants engaged in interactive, non-directive discussions, facilitated by field-specific subject matter experts and informed by their respective roles and expertise. The dialogue was structured around three core themes:
- Accessibility: assessed the timely, reliable availability of safe and effective ODs and the broader healthcare access required for drugs and care.
- Innovation: explored incentives and research priorities for developing therapies tailored to the RD patients population in Saudi Arabia.
- Sustainability and Efficiency: examined procurement mechanisms and financing models to ensure the long-term funding of ODs.
- Descriptive and thematic analyses of the discussions were conducted. The findings directly informed the development of a national strategy and a set of key policy recommendations designed to transform RDs care landscape.

RESULTS

- The workshop lasted for 8 hours with a total of 59 participants from different professional backgrounds and sectors (Table 1).

Table 1. Workshop participants characteristics

Characteristics	Participants (n)	%
Gender		
Male	37	63
Female	12	37
Age		
25-34	8	14
35-44	19	32
45-54	15	25
≥55	7	12
Professional background		
Physicians	23	39
Academics	12	20
Members of pharmacy and therapeutic committees	11	19
Clinical Pharmacists	10	17
Clinical practice guideline experts	8	14
Health authorities officials	6	10
Policy-makers	5	8
Key opinion leaders (in a specific therapy area)	5	8
Researchers/members of research agencies	5	8
Regulators	4	7
Payers	2	3
Medical insurance companies	1	2
Others	13	22

- Participants showed varied levels of understanding regarding the three themes of the study. Of the respondents, 11 described themselves as novices, 27 reported having some basic knowledge, 9 claimed a solid background, and 3 identified as experts. Nine individuals abstained from providing their level of understanding.
- The analysis of workshop categorized the identified challenges into six principal themes (Figure 1).



Figure 1. Key emerging themes.

- Through a structured voting process, participants prioritized 14 essential actions to address critical gaps in RD care in Saudi Arabia (Figure 2).

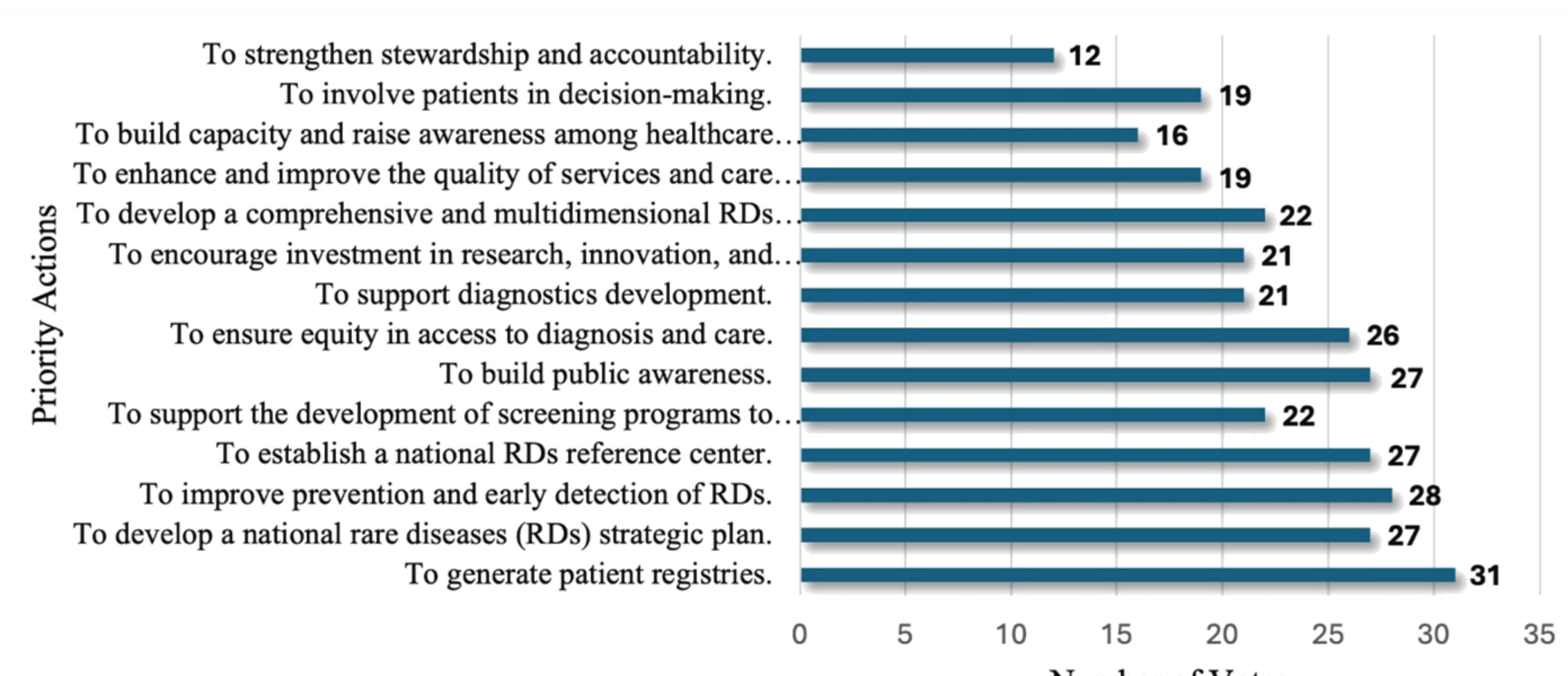


Figure 2. Prioritized areas for strategic intervention and policy development in RD.

- Workshop participants called for a series of strategic policy recommendations, notably to develop a national rare disease strategy; implement accelerated regulatory pathways; expand a national network of centers of excellence; develop a national RDs registry; and cultivate public-private partnerships.

CONCLUSIONS

- The workshop identified key priorities to strengthen the RD and OD ecosystem in Saudi Arabia, emphasizing targeted education, regulatory efficiency, patient representation, and international alignment.
- Achieving equitable access requires a multi-sectoral strategy founded on evidence-based policy, research investment, streamlined regulation, and robust advocacy.
- Implementing these key policy recommendations through sustained collaboration is essential to overcome existing challenges, enhance healthcare efficiency, and improve patient outcomes.
- Continuous engagement through regular forums is crucial for driving sustainable progress. This long-term commitment aims to position Saudi Arabia as a leader in RD care, ultimately improving the quality of life for individuals living with rare conditions.

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