Health Insurance Strategies to Improve Health Equity Among Patients with Rare Diseases in China —A Microsimulation-Based Study

RWD267

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BACKGROUND

Despite expansions in orphan drug coverage within China's basic medical insurance system, significant health equity gaps persist. Enhancing insurance coverage and benefit design for orphan drugs is crucial to improving equitable access and long-term health outcomes in rare disease treatment. Multiple sclerosis (MS), a chronic neurological disorder included in China's First List of Rare Diseases in 2018, exemplifies these challenges.

OBJECTIVE

Taking MS as an example, this study compares projected health equity outcomes and budget impact of current health insurance schemes (current policy) versus two enhanced alternatives:
(1) universal subsidies providing equal financial support for medication (universal policy);
(2) targeted subsidies offering greater support to lower-income patients (targeted policy).

METHODS

- **Data sources:** A nationwide cross-sectional online survey was conducted in July 2022. Adults (≥18 years) with a confirmed diagnosis of multiple sclerosis (MS) who provided informed consent were included. Information on demographics, baseline use of disease-modifying therapies (DMTs), and related costs was collected.
- **Prediction of DMT utilization:** A binary logistic regression model was constructed to estimate the association between annual out-of-pocket (OOP) costs and DMT use. Based on this association, and taking into account factors such as drug substitution, a prediction formula was developed using baseline utilization patterns. This formula was applied to estimate DMT utilization across income groups under three alternative scenarios.
- **Microsimulation:** A microsimulation model based on EDSS (Expanded Disability Status Scale) score was developed using a synthetic cohort of 50,000 individuals, constructed based on characteristics of the survey sample, to project quality-adjusted life years (QALYs) and costs over a 50-year horizon (figure 1). The robustness of the projections was evaluated through one-way sensitivity analyses.
- **Equity evaluation:** Equity in DMT utilization and long-term health outcomes across income groups was assessed using the generalized concentration index.

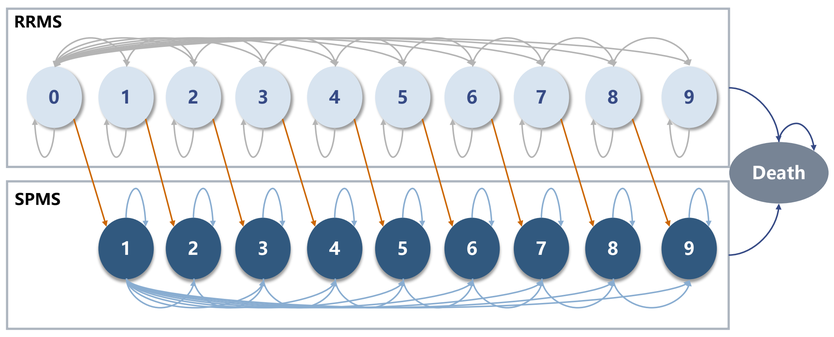

Figure 1 Microsimulation model based on EDSS score

RESULTS

- Among 658 MS patients (mean age: 34.2 years; 68.5% female), baseline DMT utilization was 61.9%, showing pro-rich inequity (GCI=0.010; 95% CI: 0.008–0.013). (Table 1)
- Each 1,000 RMB reduction in annual OOP costs increased DMT use by 0.44% (P = 0.032). Subgroup analyses across quintiles (Q1–Q5) showed corresponding increases of 0.45% (P = 0.031), 0.44% (P = 0.032), 0.42% (P = 0.033), 0.41% (P = 0.034), and 0.46% (P = 0.033). (Table 2)
- Predicted DMT utilization was highest under the targeted policy (75.5%), slightly surpassing the universal policy (75.4%), and substantially exceeding current coverage (71.0%) (Table 3). Furthermore, the utilization gap between the highest (Q5) and lowest (Q1) socioeconomic groups was reduced under the targeted policy.(Figure 2; Figure 3; Figure 4)
- Simulated QALYs averaged 6.34 (targeted), 6.33 (universal), and 6.22 (current). QALY distribution remained pro-rich under current (GCI=0.048; 95% CI: 0.025–0.070) and universal policies (GCI=0.045; 95% CI: 0.022–0.068) but equitable under targeted policy (GCI=-0.004; 95% CI: -0.027–0.019) (Table 4).
- Annual budget estimates for China's MS population were 852 million RMB (current), 979 million RMB (universal), and 972 million RMB (targeted), all demonstrating fiscal sustainability.

Table 1 DMT Utilization and disparities among 658 MS patients.

DMTs	Average utilization rate	Subgroups based on annual household income				
		Q1	Q2	Q3	Q4	Q5
Teriflunomide	27.2%	26.2%	25.0%	25.2%	29.9%	29.8%
Fingolimod	4.1%	3.4%	5.8%	5.3%	2.4%	3.8%
Siponimod	24.5%	18.8%	24.2%	33.6%	26.8%	19.8%
Dimethyl fumarate	3.5%	2.0%	3.3%	2.3%	6.3%	3.8%
Ofatumumab	2.6%	3.4%	1.7%	1.5%	2.4%	3.8%
Overall	**61.9%**	**53.7%**	**60.0%**	**67.9%**	**67.7%**	**61.1%**
GCI (95% CI)		**0.0104（0.0084，0.0125）**				

*Note: DMT, disease-modifying therapy; Q1–Q5 represent five subgroups divided by annual household income per capita from lowest to highest, where Q1 denotes the lowest 20% and Q5 the highest 20% by income; GCI, generalized concentration index; CI, confidence interval.

Table 2 The average marginal effects of annual DMT OOP on utilization, overall and by socioeconomic groups.

subgroups	Average marginal effect	SE	*P* value	95%CI
Overall	-0.0044	0.0020	0.032	(-0.0084, -0.0004)
Q1	-0.0045	0.0021	0.031	(-0.0087, -0.0004)
Q2	-0.0044	0.0021	0.032	(-0.0084, -0.0004)
Q3	-0.0042	0.0020	0.033	(-0.0080, -0.0003)
Q4	-0.0041	0.0019	0.034	(-0.0079, -0.0003)
Q5	-0.0046	0.0021	0.033	(-0.0088, -0.0004)

*Note: OOP, out-of-pocket; DMT, disease-modifying therapy; Q1–Q5 represent five subgroups divided by annual household income per capita from lowest to highest, where Q1 denotes the lowest 20% and Q5 the highest 20% by income; SE, standard error; CI, confidence interval.

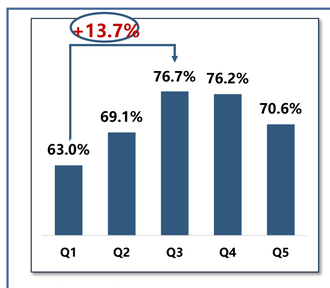

Fig.2 DMT Utilization(Current)

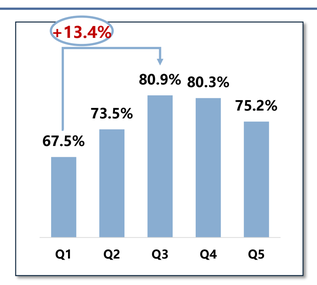

Fig.3 DMT Utilization(Universal)

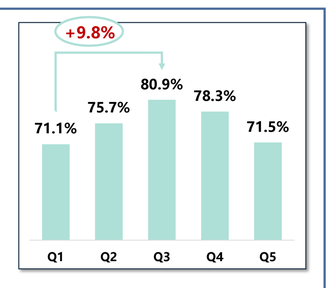

Fig.4 DMT Utilization(targeted)

Table 3 prediction of DMT utilization among 658 MS patients under different polices.

DMTs	DMT utilization rate		
	Current policy	Universal policy	Targeted policy
Teriflunomide	9.4%	10.0%	9.9%
Fingolimod	1.4%	1.5%	1.5%
Siponimod	8.4%	9.0%	9.5%
Dimethyl fumarate	26.3%	28.0%	27.8%
Ofatumumab	25.4%	27.0%	26.8%
Overall	**71.0%**	**75.4%**	**75.5%**

*Note: DMT, disease-modifying therapy.

Table 4 Disparity of QALYs gained under different policies.

Policies	GCI	95%CI
current	0.0475	(0.0248, 0.0703)
universal	0.0448	(0.0221, 0.0675)
targeted	-0.0038	(-0.0265, 0.0188)

*Note: QALY, quality-adjusted life years; GCI, generalized concentration index; CI, confidence interval.

CONCLUSIONS

- Significant health inequities exist among MS patients under current health insurance schemes.
- Targeted subsidies improve equity without compromising health outcomes or fiscal sustainability.
- Incorporating socioeconomic factors into health insurance scheme design may substantially mitigate health inequity among rare disease populations.

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