PARIS SCHOOL OF ECONOMICS
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Working paper

"Adopting a universal care pathway in rare disease"

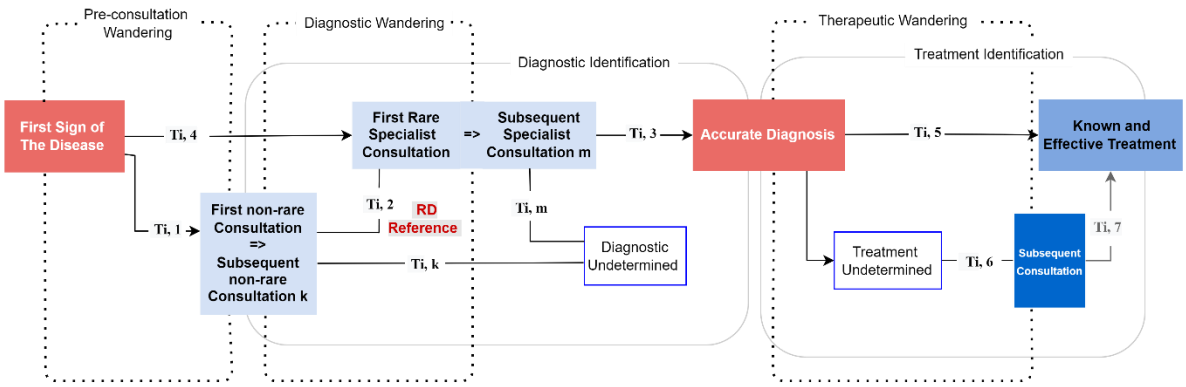

Figure: The universal framework of the care pathway of patients with a rare disease

All defined rare diseases have in common the characteristic of low prevalence, high complexity, and large heterogeneity. Encountering a patient with rare condition in the healthcare system is not uncommon. Patients often live with a rare disease for many years without knowing what the root of their affliction is, uncertainties may lead to anxiety and even fear about learning the conditions. In general, challenges appear all along the care path and are faced by all participants of the healthcare system ranging from patients, caregivers, and practitioners to policymakers, private industry players and researchers. It is needed to enact strategies that will harmonise perspectives and ensure the efficiency of the collective's efforts.

For quantitative research on rare diseases, inferring the start and end of time to diagnosis can be a challenge, due to the lack of standards of consensual definitions of the measures and potential common medical procedures followed by the patients. We can generalise and simplify the healthcare pathways of many patients with a rare disease using a pragmatic tree-like graph as shown. This graph is made up of 8 nodes connected by 10 edges, for which 6 edges are oriented from left to right and the rest edges are undirected (can go both ways). Each node represents a defined critical stage that could be a care event or a clinical status, and the lengths/weights of the edges indicate the time durations between the connected two nodes. From a macro level, this graph mimics the workflow in the healthcare system, such that patients can enter, pause, and exit the care at any stage, following a predefined direction from the left to the right. The framework, therefore, has the capacity to trace a large variety of unique care paths for different combinations of care events and time duration that patients

have experienced. It helps reveal the multiple dimensions of patient care paths, and allows for research across time, stage, disease, and individual.

We define the framework over three main phases during which wandering may take place: (1) a pre-consultation wandering phase, (2) a diagnostic identification phase that covers the diagnostic wandering, and (3) a therapeutic wandering phase. There are many causes of delays at each defined stage from the demand side (patients) and the supply side (the healthcare systems). The duration of the pre-consultation wandering phase relies on patient self-appraise of their bodily changes, which is subjective. It is also found to be related to individual health behaviours that are influenced by social stigma and can be determined by socio-economic status. The diagnostic identification phase is considerably complex as various parties will interact and jointly make decisions during this phase. Therefore, this period accounts for a substantial portion of the delay in a patient's care journey. Finally, during the therapeutic wandering phase, treatment-related delays are largely attributed to the availability of and accessibility to treatments, which is inherently related to pharmaceutical R&D and clinical trials.

To conclude, understanding the mechanisms in which the delays were influenced by each factor is challenging and data demanding yet essential. This framework could facilitate system-level organisational improvements in the short term, provide health professionals with a fresh perspective using analytical evidence, which ensures a longer-term benefit including patient health and satisfaction associated with shorter time to diagnosis, high overall social value of health care services. The results from this approach could also advice and inform policymakers that wish to develop effective policies in reducing diagnostic delays in rare diseases.

Biography:

Meng Jiang is a Ph.D. student from Paris School of Economics and the university Paris 1 Panthéon-Sorbonne. She is a health economist who has background in Economics, Data Sciences, and Finance. Her past work has focused on improving equity and efficiency in rare diseases, using the methods from behavioural and experimental economics and economic theory. Her current studies will shed light on the construction of a generic care pathway, eliciting preferences on health information using experiments, and measuring R&D spillovers cross rare and non-rare drugs.

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