Developments in genomics and the shift towards personalised medicine is:
§ resulting in the stratification of diseases into smaller subsets and therefore into smaller population sets;
§ reclassifying diseases under a new taxonomy; and
§ is leading to an increasing body of diseases that qualify as rare.

The current regulatory model:
§ does not distinguish between the pre- and post-genomic paradigm of diseases; and
§ is market oriented, where industry can select which orphan drugs are prioritised.

Recognising that the basic needs of rare diseases patients were not being met, the EC Orphan Drug Regulation (No. 141/2000) was established in 2000. It:
§ establishes a Community procedure for the designation of medicinal products as orphan medicinal products;
§ adopts a rights-based approach, regulating and promoting innovation in the field of orphan drugs; and
§ is incentive based.

A successful designation is granted based on:
§ prevalence of the rare disease (5 out of 10,000 individuals or less);
§ the seriousness of the disease;
§ an unmet need; as well as
§ potential benefit.

Such barriers have resulted in injustice and inequity where the medical and social needs of rare disease patients have often gone unmet.

Rare disease patients have faced additional obstacles in accessing health care services and treatment compared to patients with common diseases, including:
§ a lack of access to and delays in a correct diagnosis;
§ a lack of adequate information and support;
§ a lack of appropriate treatment or alternatively high costs for existing treatment plans; and
§ a lack of specialised social services.

Analysing the Effects of Personalised Medicine on the EU Regulatory Framework for Orphan Drug Research and Development
Jasjote Grewal

Background
Rare disease patients have faced additional obstacles in accessing health care services and treatment compared to patients with common diseases, including:
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EU Regulatory Framework
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Future Challenges
Developments in genomics and the shift towards personalised medicine is:
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Approach
The intersection between the EU regulatory framework for orphan drugs and personalised medicine will be explored as follows:
§ Legal Analysis: EC Orphan Drug Regulation (No. 141/2000), with an impact assessment on the status quo of the current governance framework;
§ Qualitative Methodology: Semi-structured expert interviews; and
§ Normative Analysis: Based on empirical findings.

Publications on the analysis of the effects of personalised medicine on the EU regulatory framework for orphan drug research and development;
Recommendation paper for future orphan drug policy in light of the increasing trend towards personalised medicine; and
Presentation on the future of the EU orphan drug regulatory framework in the context of advances in genomic technologies.