Therapeutic Individualization - Clinical Pharmacology in Addressing Specific Populations

FDA/MCERSI Hybrid Workshop: Clinical Pharmacology Guidances Advancing Drug Development and Regulatory Assessment: Role and Opportunities

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Elimika Pfuma Fletcher, PharmD, PhD
Policy Lead
U.S. FDA/ CDER/OTS/OCP

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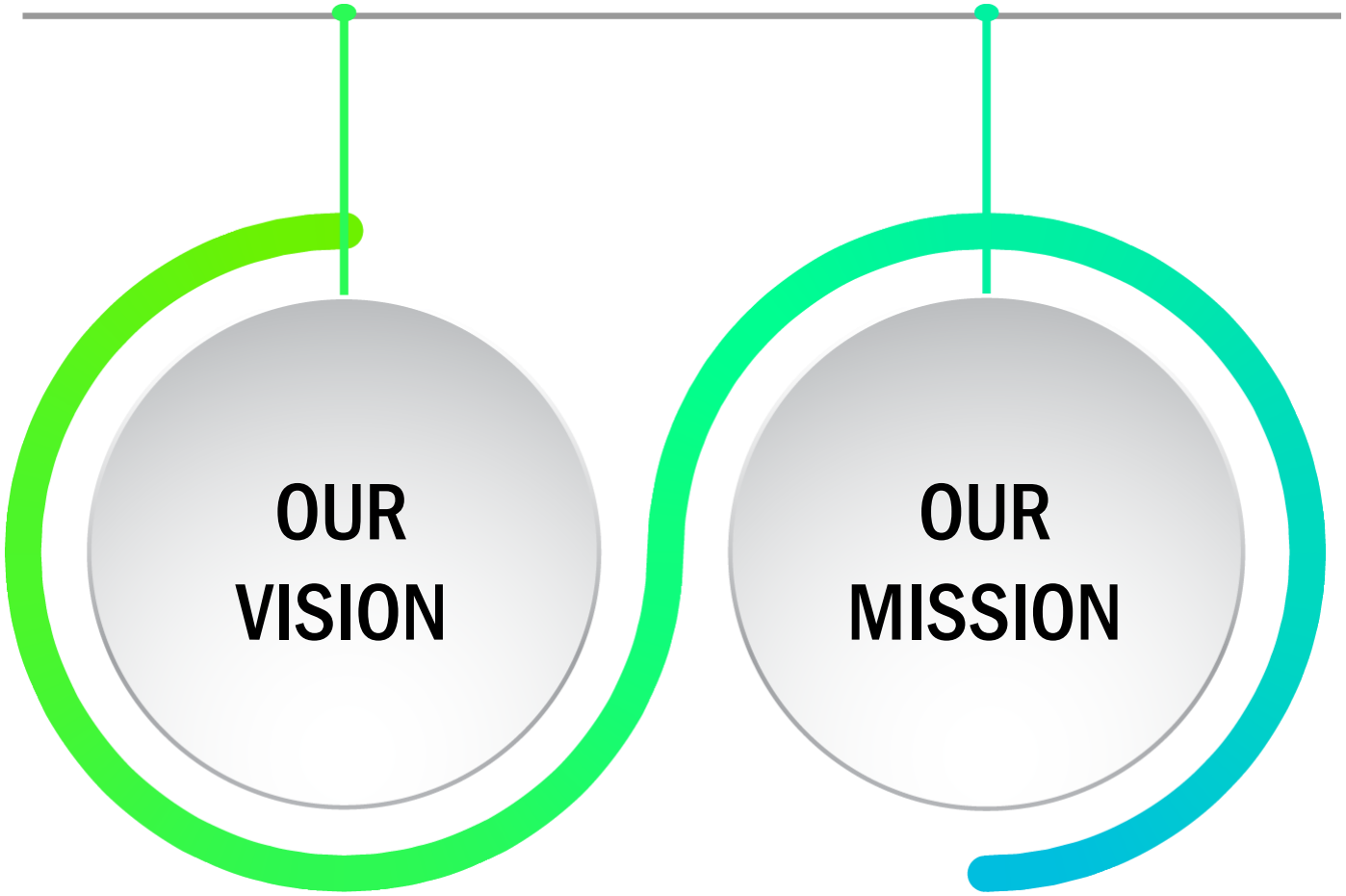
Office of Clinical Pharmacology (OCP)

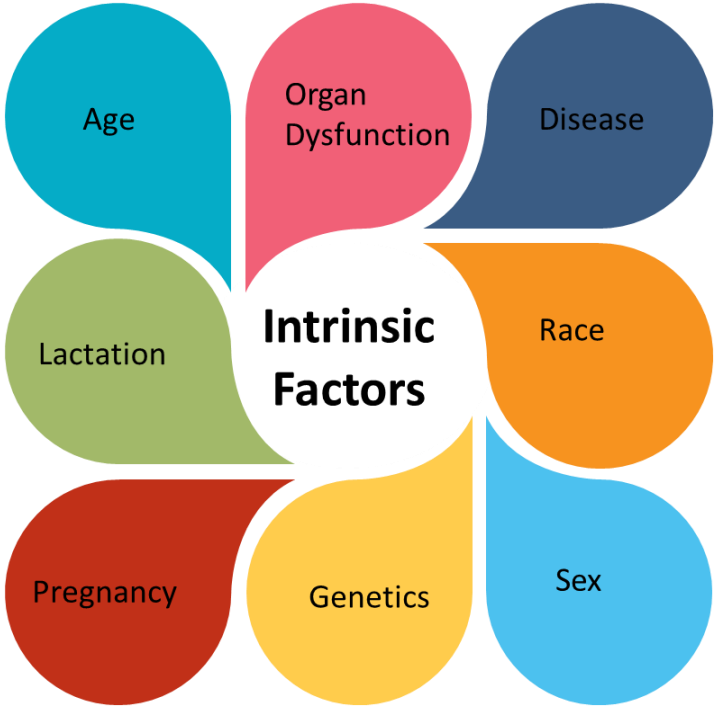
OCP is a dynamic, purpose-driven organization whose goals are to enhance drug development, promote regulatory science and innovation, and infom the optimal use of medications.

Improve public health by building and translating knowledge of drug response into patient-centered regulatory decisions of the highest quality



- Play a pivotal role in advancing development of innovative new medicines by applying state-of-the-art regulatory science and clinical pharmacology principles
- Promote **therapeutic optimization and individualization** through best practices in research, policy development, and drug evaluation throughout the product lifecycle

Why Individualization



Not all patients with the same diagnosis respond the same to treatment

Differences in intrinsic and extrinsic factors can result in differences in efficacy and/or safety

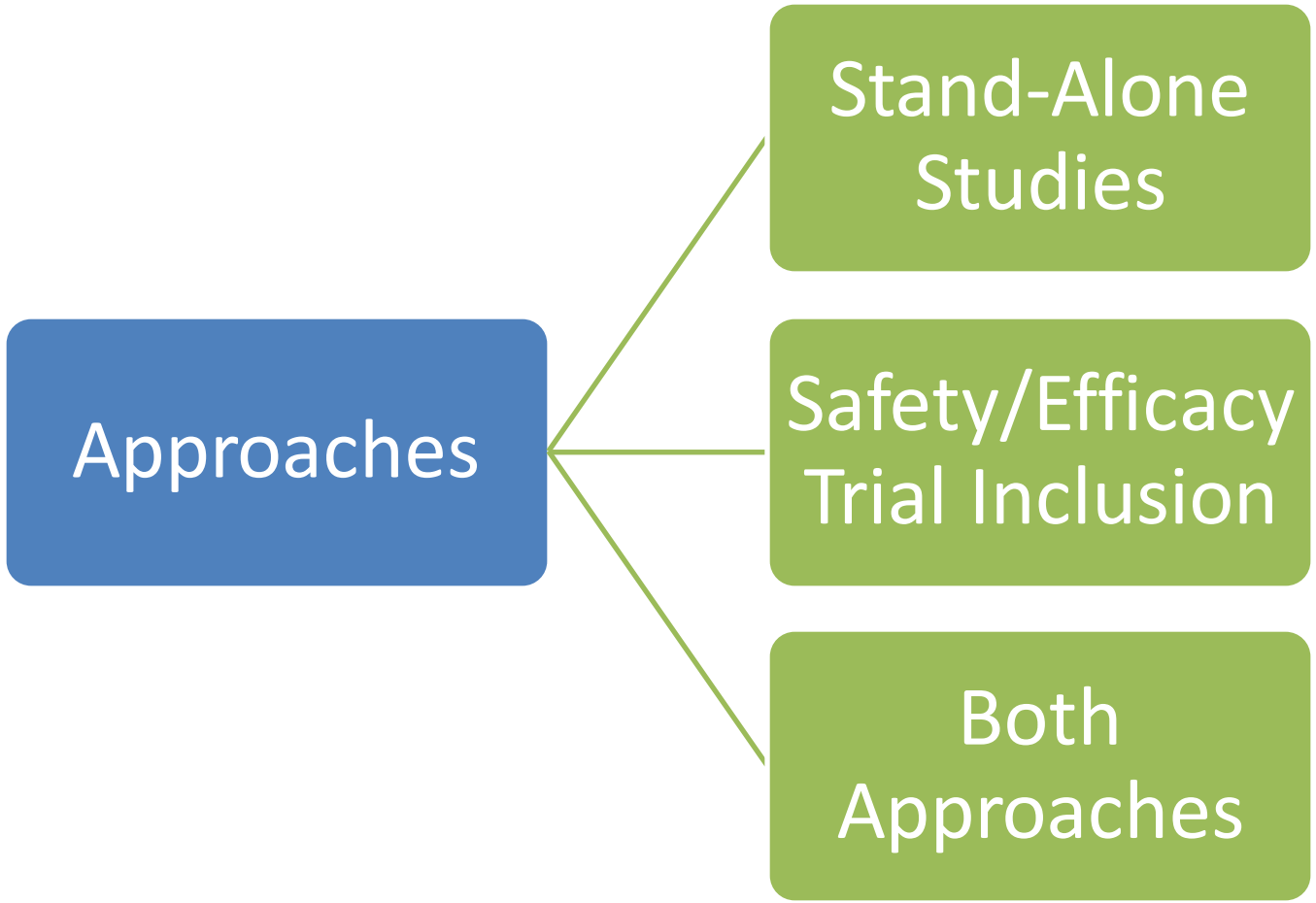
Design Source: presentationgo and OCP StratComm

***Goal of Diversity Initiatives**

Right drug, for the right person, at the right dose

Source: Anu Ramamoorthy

How are Specific Populations Studied?

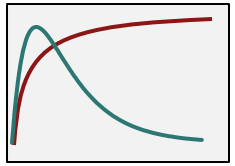


- PK and/or PD considered in making recommendations in specific populations
- Exposure matching generally used to inform dosing - assumes similar PK/PD relationship

Source: Madabushi R Workshop Slides

Clinical Pharmacology Assessment: Key Questions

Comprehensive Clinical Pharmacology Review Questions



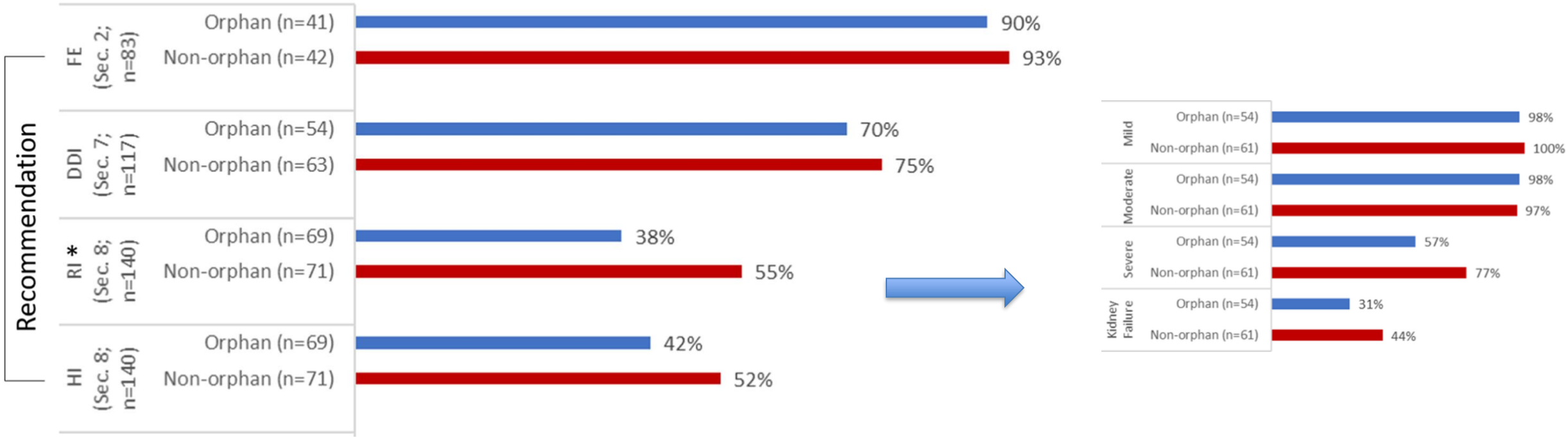
To what extent does the available clinical pharmacology information provide pivotal or supportive evidence of effectiveness?

Is the proposed dosing regimen appropriate for the general patient population for which the approval is being sought?

Is an alternative dosing regimen or management strategy required for subpopulations based on intrinsic factors?

Are there clinically relevant food-drug or drug-drug interactions, and what is the appropriate management strategy?

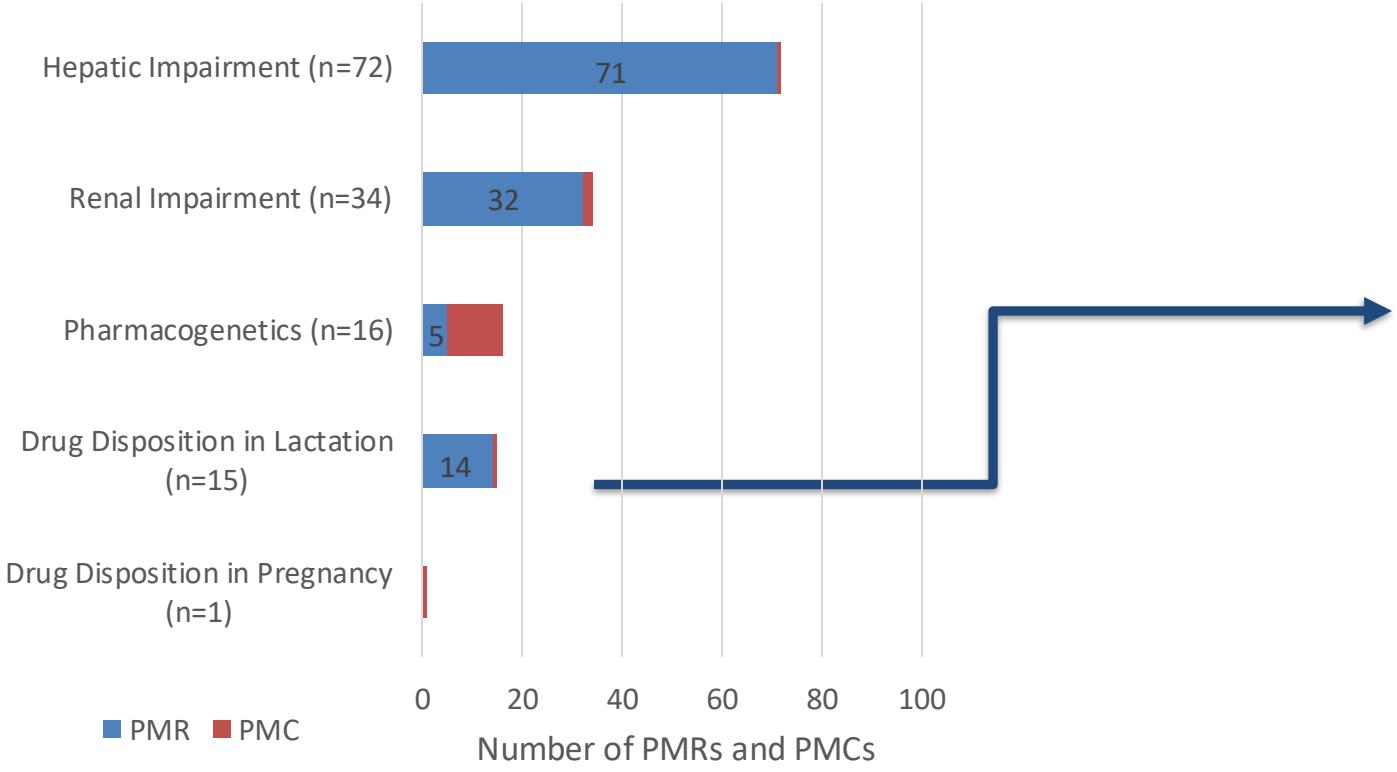
Intrinsic/Extrinsic Factors in Labeling

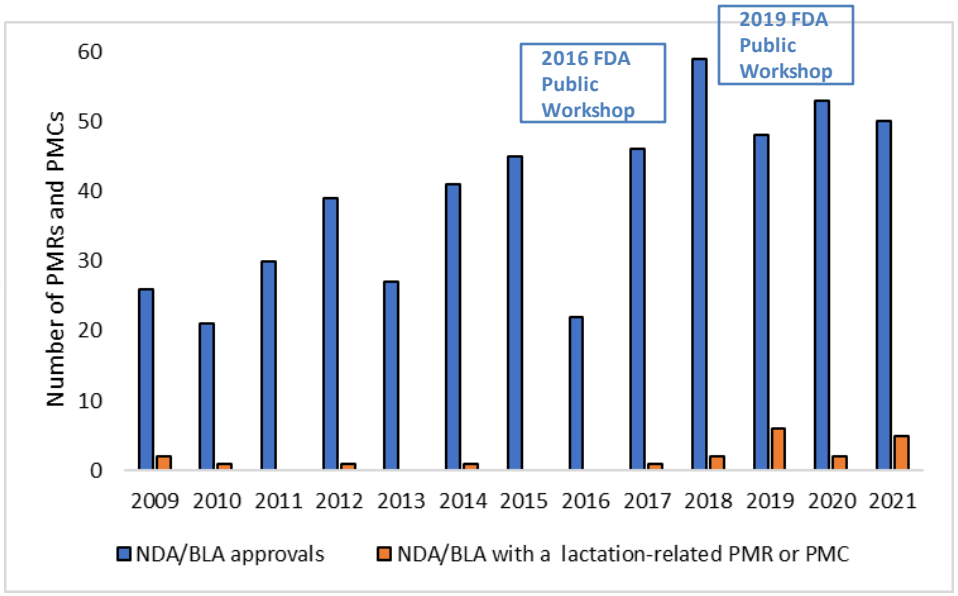


Based on evaluation of 153 NMEs approved by the CDER from 2017 to 2019

Hsieh J et al. (2022)

Postmarketing Studies (Intrinsic Factors)

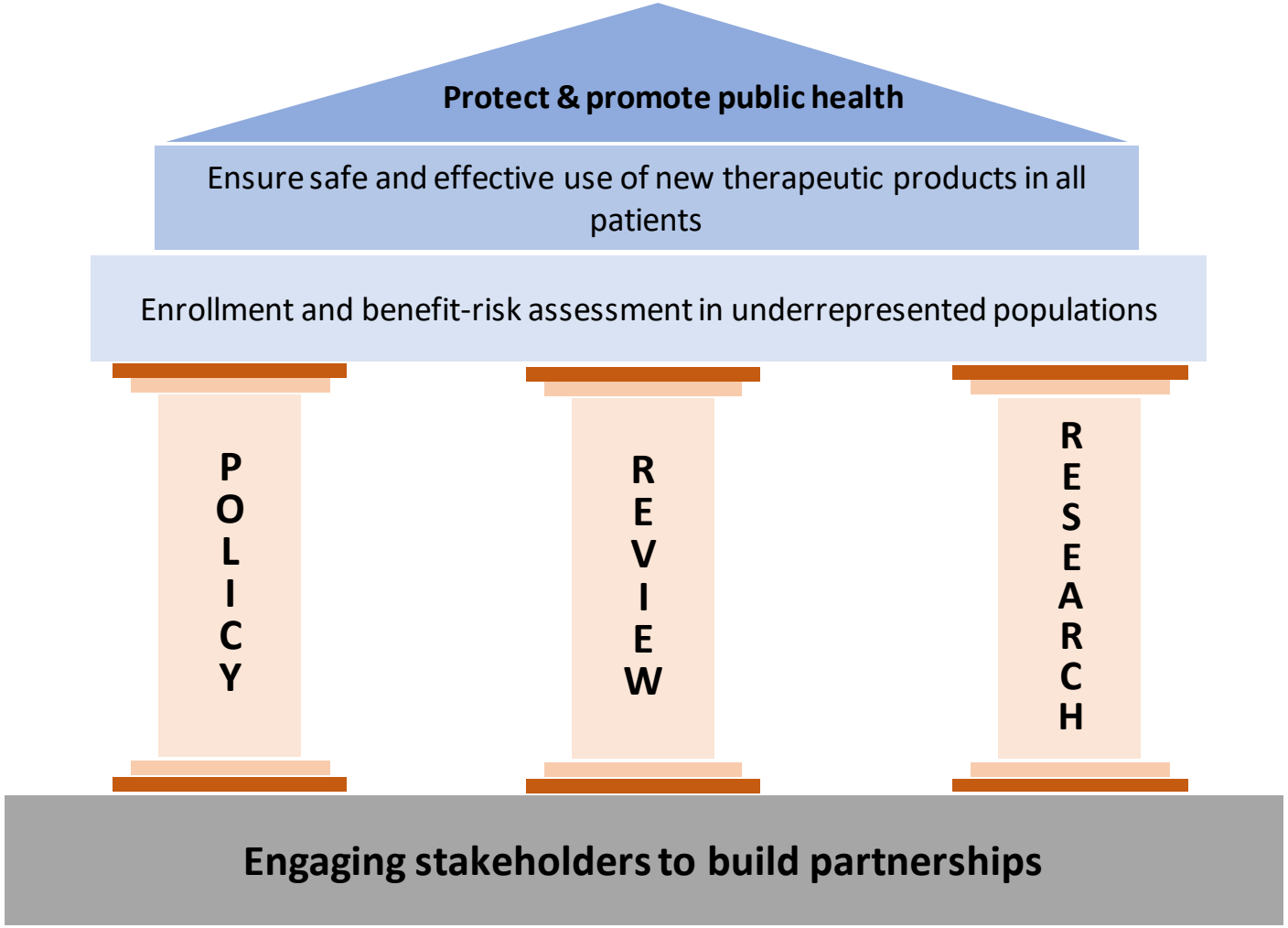




PMR/PMC for NMEs approved 2009 - 2020

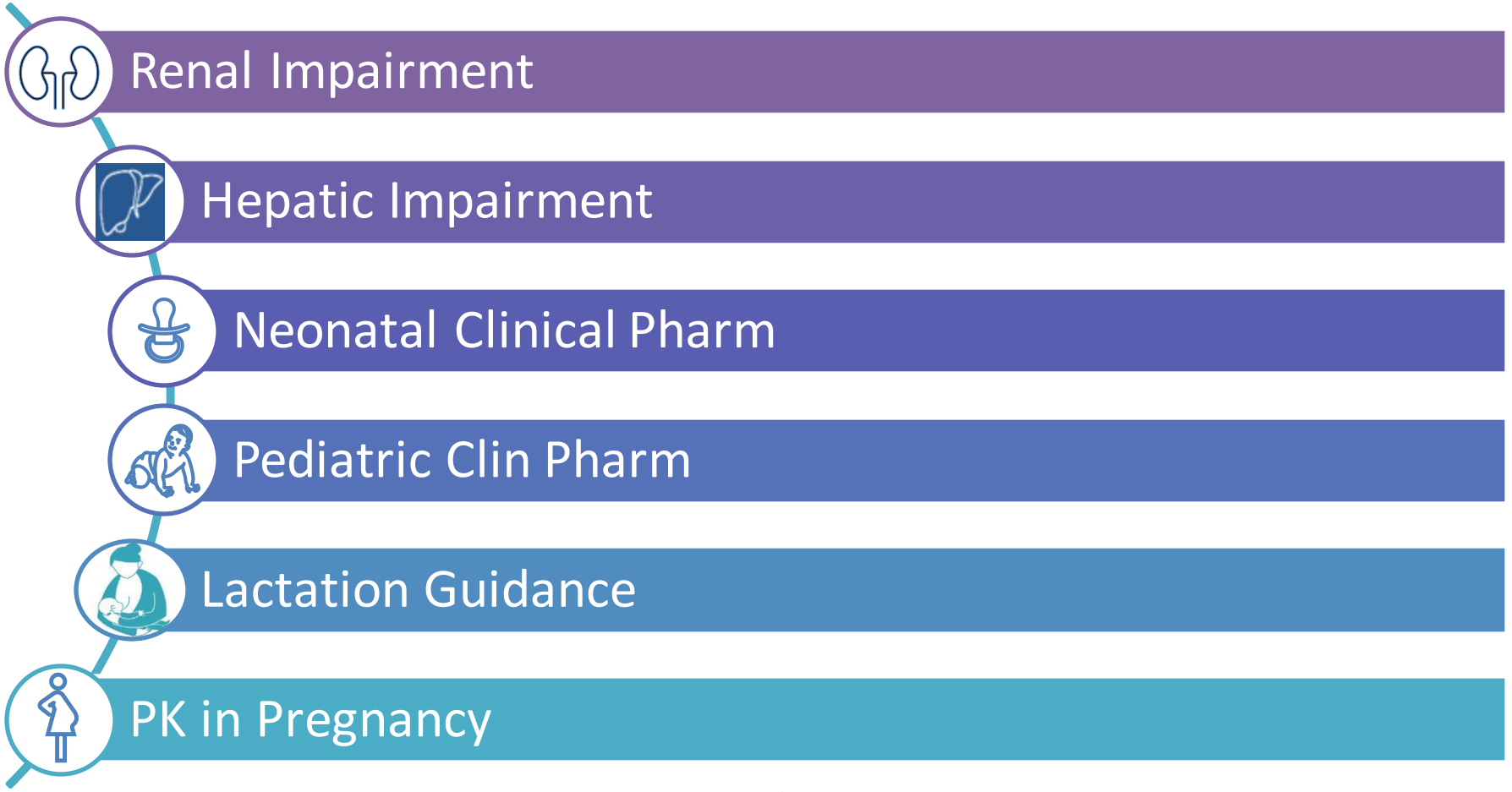
Source: Ridge S. et al. (2023) and Ridge S et al. ASCPT Poster (2023)

Promoting Therapeutic Individualization



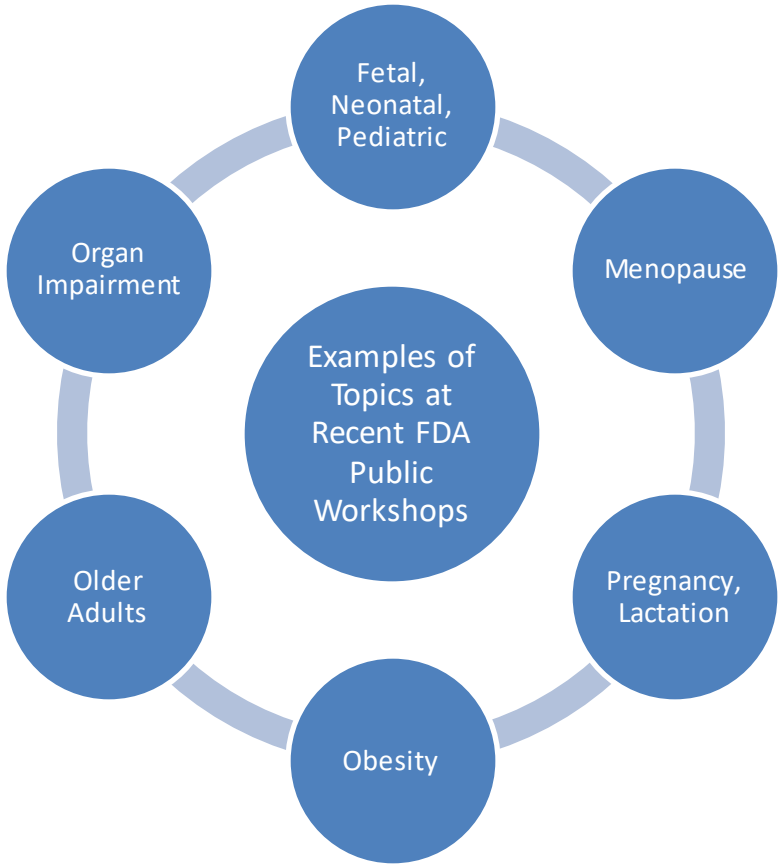
Ramamoorthy et al. (2022)

Examples of Guidances Documents



- Some broader guidances address intrinsic factors e.g. older adult guidances
- Several therapeutic area specific guidances address intrinsic factors e.g pediatrics
- Supported by legislative initiatives (e.g. FDORA- Diversity Action Plans)

Stakeholder Engagement



- Additional clinical pharmacology discussions in FDA workshops on therapeutic specific or broader topic (pediatric, maternal, diversity, RWE)
- Additional engagement in professional societies and other venues

Summary

- The mission of OCP is to advance development of innovative new medicines and promote therapeutic optimization and individualization

- OCP works towards its mission and goals through regulatory review, policy, regulatory research and stakeholder engagement

- In the current session, stakeholders will provide input on potential opportunities and priorities in addressing specific populations

Acknowledgements

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