Patient and Community Engagement throughout the Health Product Lifecycle- Towards Sustainable Partnership for Access

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Disclaimer: this presentation is in the presenter's own personal capacity and is not necessarily representative of the position of Takeda Pharmaceuticals International AG

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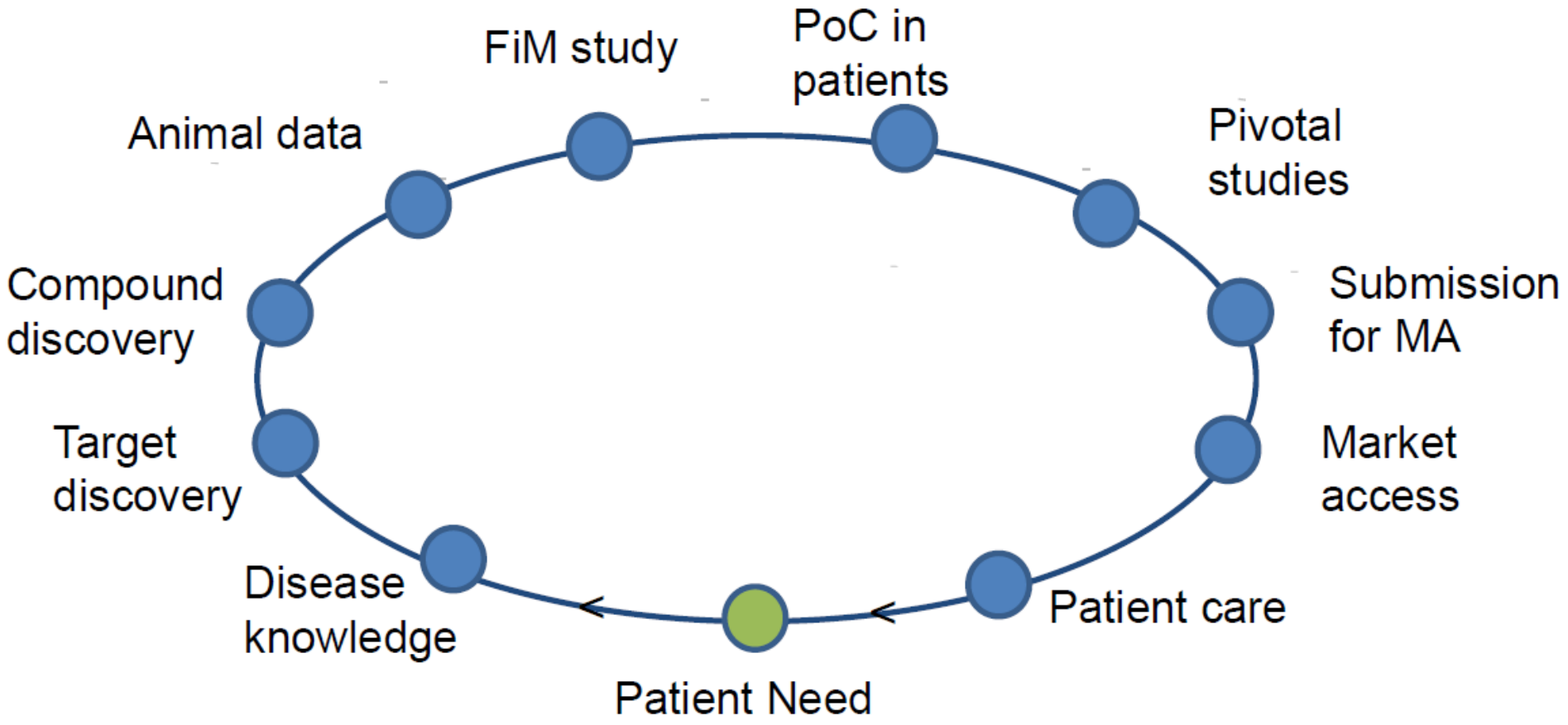
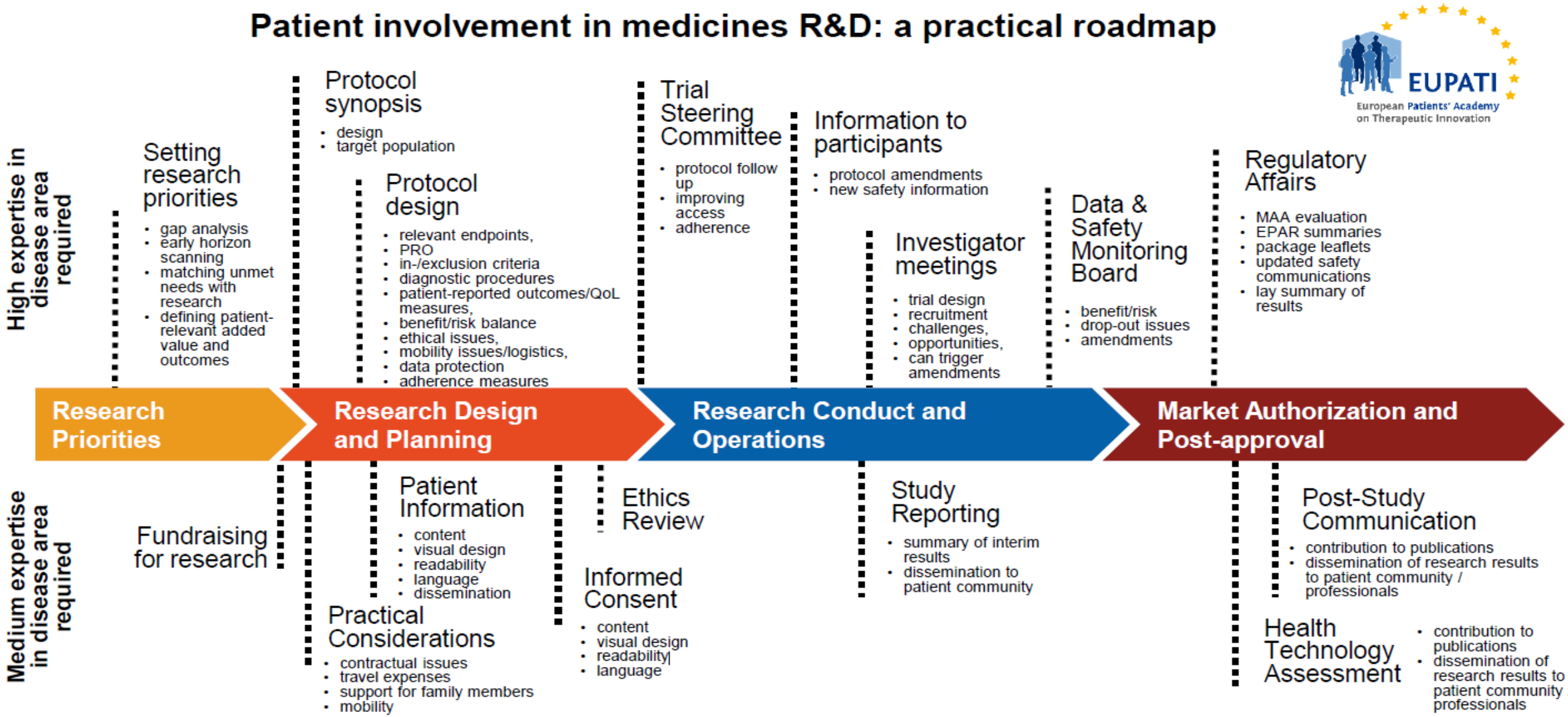
The journey from molecule/concept to medicine/device starts with patients



Modified from IRDIRC “Orphan Drug Development Guidebook” IRDIRC “Orphan Drug Development Guidebook” : https://orphandrugguide.org/

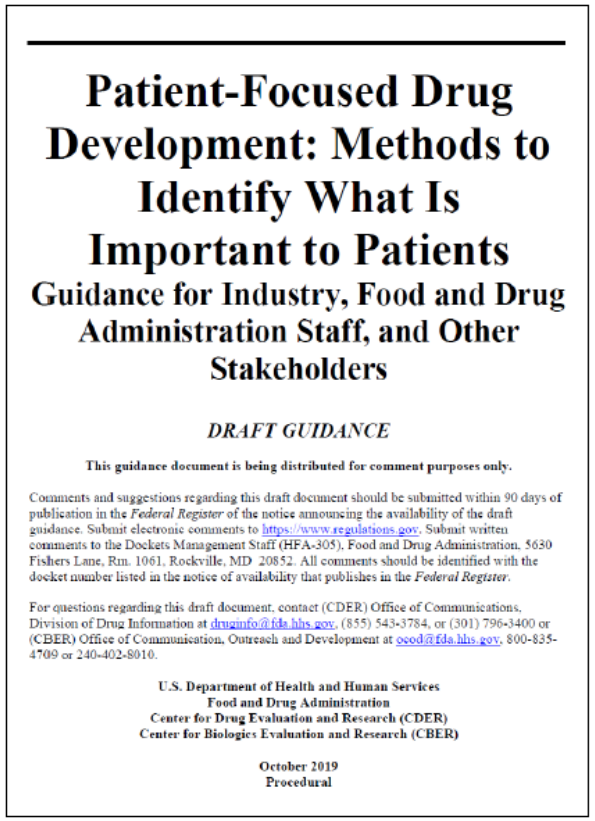
Patients add value at every stage of the product development lifecycle



Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

Patient Engagement is now a “must-have”

Patients are more knowledgeable and demanding of their human rights



Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

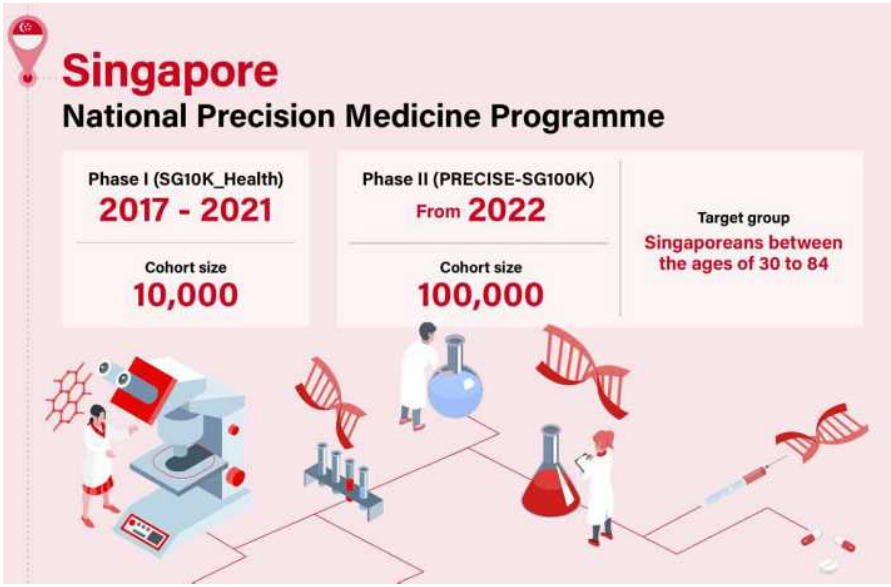
Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Office of Communications, Division of Drug Information at druginfo@fda.hhs.gov, (855) 543-3784, or (301) 796-3400 or (CBER) Office of Communication, Outreach and Development at ocod@fda.hhs.gov, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

October 2019
Procedural

Patient-focused trial outcomes and measures are now recommended or even required by major regulatory authorities and HTA bodies



Shift to personalised healthcare will necessitate more involvement of patients and the public

e.g. (ethical considerations for genomic data, small (n=1) trials, biomarkers)

Collaboration is a win-win for patients and health product researchers

Benefits to patients

- Patients' real needs are being met (they know them best)
- Trial endpoints relevant to patients
- Feasible study protocols
- Faster availability of new health products

Benefits to researchers

- More accurate identification of unmet needs
- Relevant patient-reported outcomes
- Faster conduct of trials and increased retention and positive experience
- Quicker path to market

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Patient engagement has real and measurable impact on health product development

- Patient voice had lasting changes in regulatory procedures e.g. expanded access/compassionate use (FDA 1987), accelerating approval life-threatening conditions (FDA 1992), use of surrogate markers instead of clinical markers in pivotal trials (EMA, NVP approval 1997)
- New criteria for conditional approval (EMA, 2006)
- Lazarus effect on dying patients & HIV cohort studies in place to prove cost-effectiveness of expensive treatment
- Cross-Atlantic lobbying for pivotal trial including 2 new chemical entities(NCE), reducing exposure to monotherapies & multidrug resistance (2007)
- Single tablet regimens for convenience and adherence, while having single compounds to control toxicities, resistance and adapt drug levels, FDA: 27 NCE & 14combos (1987-2017)

Patient engagement has real and measurable impact on health product development

- Cystic fibrosis – personalized medicine: approval of mutation specific disease modifying therapy for incurable genetic disease with support of Cystic Fibrosis Foundation
- Therapy development in rare diseases is driven by patient organizations
- Patients have shifted from protest-led approaches and have moved towards becoming partners
  - Patients are members at FDA/EMA governance and scientific committees
  - Many patient community advisory boards have been introduced pioneered for HIV trials

Enablers for enhancing patient engagement

- Increasing awareness of general public (who may not be personally affected by certain health conditions) on importance of trials and medicines development, regulation and access

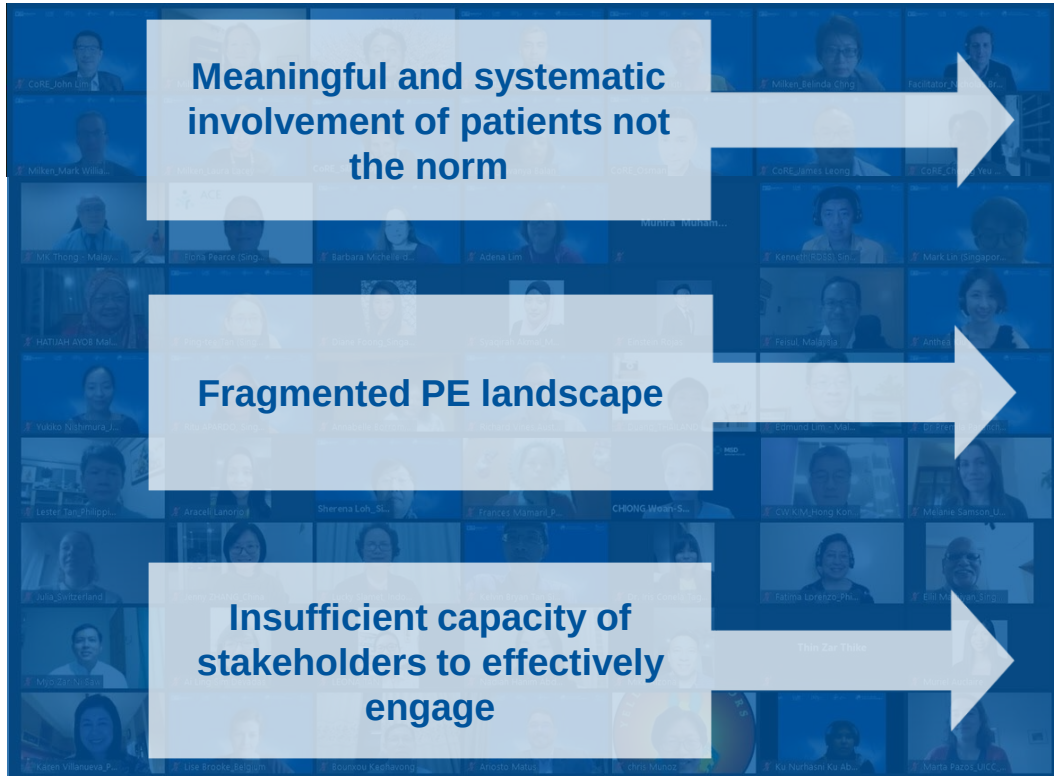
- Political and organizational will to institutionalise patient input to avoid “token” involvement – investment from diverse sources needed to sustain inclusive platforms and build capacity in cross-sector engagement e.g. EU-IMI funding

- Capacity-building of patient advocates to equip a critical mass with sufficient knowledge on technical aspects of health products development to give meaningful contributions

- SMART evaluation of patient engagement to document the measurable impact

CAPE – A Singapore-grown vision for Patient Engagement in Asia

Patients as Partners for Sustainable Health Systems



Strong person-centred health systems

Multi-stakeholder communication & coordination

Empowered stakeholders with tools and skills for patient engagement

CAPE

Coalition to Accelerate Patient Engagement in the Asia-Pacific

Inspiration from the region

- **Patient and Public Involvement Japan** - Multistakeholder platform in Japan bringing together all relevant stakeholders in the medicines development ecosystem

- **The Consumer Education and Engagement (CEE)** initiative at Singapore's HTA body Agency for Care Effectiveness

- **Clinical Research Malaysia** runs a clinical trials awareness campaign called " I Am Aware" that includes multimedia promotion through social media, TV and radio as well as roadshows across the country

- **KoNECT South Korea** runs national campaigns educating and encouraging patients to participate in trials through real-life patient stories

Some Resources

- More about the Coalition to Accelerate Patient Engagement in Asia-Pacific (CAPE): https://www.duke-nus.edu.sg/core/think-tank/coalition-to-accelerate-patient-engagement-in-asia-pacific-(cape)-strengthening-health-systems-oriented-to-patient-needs
- CIOMS Working Group recommendations on patient involvement in the development, regulation and safe use of medicines : https://cioms.ch/publications/product/patient-involvement/
- PPI Japan - Multistakeholder platform in Japan bringing together all relevant stakeholders in the medicines development ecosystem : https://www.ppijapan.org/
- The Consumer Education and Engagement (CEE) initiative at Singapore's HTA Agency ACE : https://www.ace-hta.gov.sg/Patients-And-Community/cee-updates
- PFMD Global Patient Engagement and Patient Experience Data project: https://patientfocusedmedicine.org/patient-experience-data/\
- IMI-EU PARADIGM project : https://imi-paradigm.eu/our-approach/
- European Patient Academy of Therapeutic Innovation: https://eupati.eu/

Thank You!