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PERSPECTIVE

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How can we deliver on the promise of precision medicine in oncology and beyond? A practical roadmap for action

Anne-Marie Baird[1,2] | C. Benedikt Westphalen[3] | Sandra Blum[2,4] | Begonya Nafria[2,5,6] | Tanya Knott[2,7] | Ify Sargeant[8] | Helena Harnik[2,9] | Nicholas Brooke[2,9] | Nicole Wicki[2,9] | Durhane Wong-Rieger[2,10]

[1]Lung Cancer Europe (LuCE), Bern, Switzerland

[2]From Testing to Targeted Treatments (FT3) Program Team, The Synergist, Brussels, Belgium

[3]Comprehensive Cancer Center Munich and Department of Medicine III, University Hospital, LMU Munich, Munich, Germany

[4]Roche, Basel, Switzerland

[5]Institut de Recerca Sant Joan de Déu, Barcelona, Spain

[6]Innovation and Research Department, Hospital Sant Joan de Déu Pg, Barcelona, Spain

[7]Sarah Jennifer Knott (SJK) Foundation, Dublin, Republic of Ireland

[8]Twist Medical, Burlingame, California, USA

[9]The Synergist, Brussels, Belgium

[10]Canadian Organization for Rare Disorders, Toronto, Ontario, Canada

Correspondence

Helena Harnik, From Testing to Targeted Treatments (FT3) Program, The Synergist, Ave Louise 231, 1050 Brussels, Belgium.
Email: helena@thesynergist.org

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Abstract

Background: Precision medicine (PM) is a form of personalized medicine that recognizes that individuals with the same condition may have different underlying factors and uses molecular information to provide tailored treatments. This approach can improve treatment outcomes and transform lives through favorable risk/benefit ratios, avoidance of ineffective interventions, and possible cost savings, as evidenced in the field of lung cancer and other oncology/therapeutic settings, including cardiac disease, diabetes, and rare diseases. However, the potential benefits of PM have yet to be fully realized.

Discussion: There are many barriers to the implementation of PM in clinical practice, including fragmentation of the PM landscape, siloed approaches to address shared challenges, unwarranted variation in availability and access to PM, lack of standardization, and limited understanding of patients' experience and needs throughout the PM pathway. We believe that a diverse, intersectoral multistakeholder collaboration, with three main pillars of activity: generation of data to demonstrate the benefit of PM, education to support informed decision-making, and addressing barriers across the patient pathway, is necessary to reach the shared goal of making PM an accessible and sustainable reality. Besides healthcare providers, researchers, policymakers/regulators/payers, and industry representatives, patients in particular must be equal partners and should be central to the PM approach—from early research through to clinical trials and approval of new treatments—to ensure it represents their entire experience and identifies barriers, solutions, and opportunities at the point of delivery.

Conclusion: We propose a practical and iterative roadmap to advance PM and call for all stakeholders across the healthcare system to employ a collaborative,

cocreated, patient-centered methodology to close gaps and fully realize the potential of PM.

KEYWORDS
collaborative precision medicine, equitable access precision medicine, patient engagement, precision medicine

1 | INTRODUCTION

The term "personalized medicine" has a broad definition that incorporates many factors; in general, it refers to treatment that is tailored to an individual's characteristics taking the approach that patients even with the same disease can have different underlying factors. Precision medicine (PM) is a type of personalized medicine that aims to achieve improved outcomes by providing more effective treatments and interventions based on molecular information (genomic, transcriptomic, proteomic, and metabolomic) that can help determine molecular drivers of disease in each patient (PM; https://efpia.eu).[1–7] PM approaches can potentially deliver several benefits for patients and healthcare systems, such as improved treatment efficacy by reducing trial and error prescribing and ineffective interventions, leading to a favorable risk/benefit ratio; reducing the occurrence and magnitude of adverse drug reactions; reducing high-risk invasive procedures; and increasing treatment adherence.[8–12] This ultimately drives a shift toward patient-centered care and leads to possible healthcare cost savings.

Proof of concept for the potential of PM is best demonstrated in oncology, where there has been unquestionable improvement in outcomes following targeted therapies for people (who have access to testing and appropriate treatments) with nonsmall cell lung cancer whose tumors harbor targetable mutations.[13–15] Therefore, we focus primarily on oncology as an example to demonstrate the challenges around PM. The application of molecularly driven PM has expanded beyond lung cancer, with first examples in hard-to-treat cancers such as cholangiocarcinoma or genomically defined subtypes of solid (e.g., prostate, breast) and hematological (e.g., chronic myeloid leukemia) cancers.[16] This demonstrates that PM can transform lives by helping to provide a better quality of life, as well as more efficient and safer treatments. With the integration of advanced technologies, including artificial intelligence, machine learning, and micro/nanorobots, PM will increase in both scope and precision capabilities to evolve into the standard of care in more diverse cancers.[17,18] However, the scope and potential benefits of PM have not yet been fully realized. Here, we outline the rationale for an approach to PM that brings together all stakeholders across the healthcare system to encourage a collaborative, cocreated, patient-centered methodology. It is our belief that this is the only way that PM will become an accessible and sustainable reality for everyone who could benefit from it.

2 | WHY IS A NEW APPROACH TO PM NEEDED?

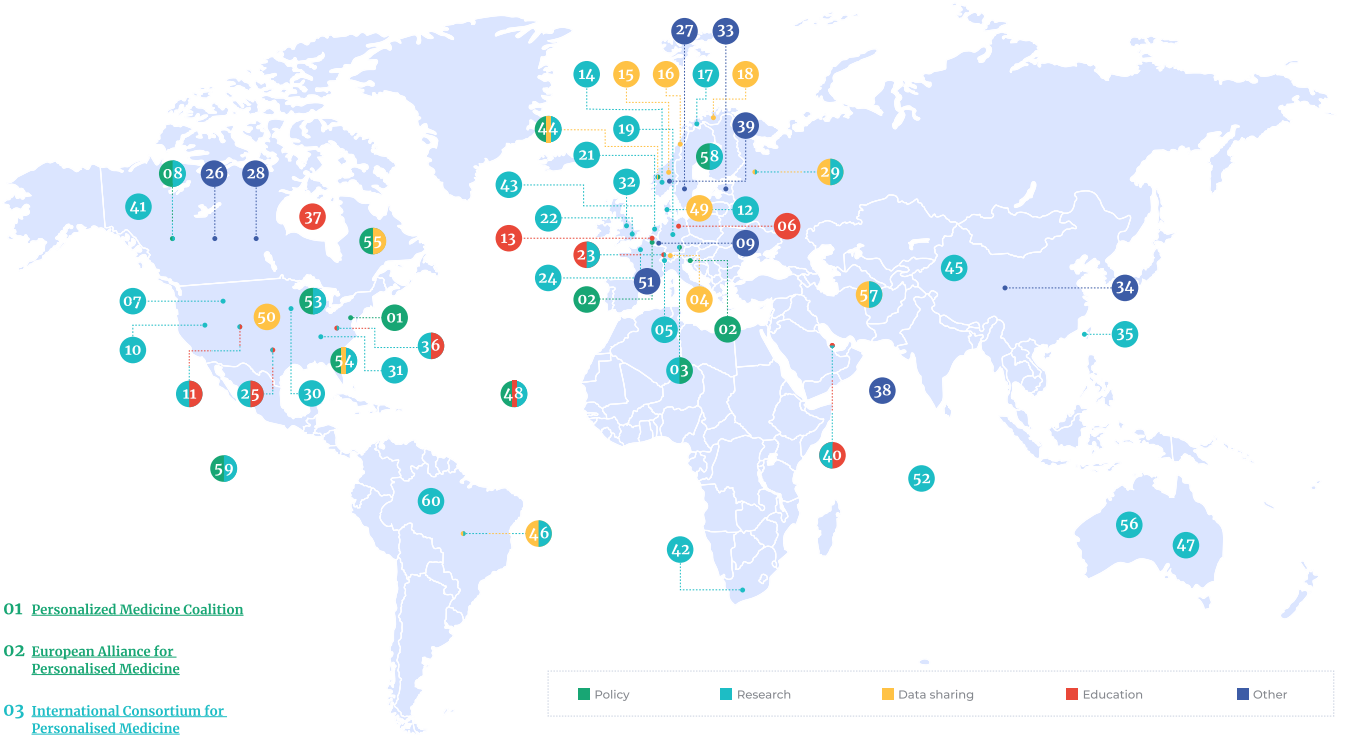
Challenges and barriers to the implementation of PM in cancer clinical practice exist across the continuum of the patient pathway and different stakeholder groups (Figure 1).[12,19,20] In reality, the time for cancer clinical practice to evolve toward PM has passed; PM is already embedded and driving diagnosis and treatment across multiple disease areas. In cardiac disease, the discovery of gene mutations related to high cholesterol led to targeted therapeutics, a move that has been dubbed a "paradigm shift" in cholesterol treatment.[21] The Multinational Precision Medicine in Diabetes Initiative, formed to advance the application of PM into the "phenotype known as diabetes," will build upon the new diagnostic and therapeutic pathways identified for single-gene variants as well as complex type 2 diabetes.[22] Similarly, the Alzheimer Precision Medicine Initiative is coordinating the "conceptual shift from ineffective treatments for biologically heterogeneous 'population averages' to individually tailored biomarker-guided targeted therapies."[23] In rare diseases (e.g., inborn errors of metabolism, cystic fibrosis, spinal muscular atrophy, hemophilia), the ability to identify highly specific genomic variants is creating a "paradigm shift" by matching individuals to targeted therapies and therapeutic combinations.[24–28] Despite these advances, there is little coherence among PM initiatives; consequently, each challenge is likely to be addressed by several people and organizations across various regions and conditions, all with potentially different priorities. It is possible for organizations to work together, however. For example, patient organizations such as LUNGevity in the United States[29] and Lung Cancer Europe (LuCE)[30] run initiatives that include education, research, and support for people impacted by lung cancer. Patient-led oncogene groups are also active in providing education as well as promoting and funding specific oncogene-driven research.[31] Such groups have similar aims, challenges, and solutions, which means they can work collaboratively, thereby synergizing efforts for maximum impact.

Challenges to PM can only be overcome with solution-driven, innovative thinking and through balanced cocreation and collaboration among all healthcare system stakeholders, including healthcare providers (HCPs), researchers, policymakers/regulators/payers, and industry representatives, and importantly, patient communities. All parties must come together, irrespective of disease type, to ensure a strong patient voice in existing and fledgling initiatives. This requires more education and awareness of PM within patient communities. It is also critical that the patient voice is present from the outset, that patients champion and help to advance the PM agenda, and that they have the necessary tools and resources. PM has been advanced by patient involvement, for example,

FIGURE 1 Schematic of a patient pathway in lung cancer outlining the barriers to implementing diagnostic biomarker testing at each stage of the pathway for the different stakeholder groups: HCPs, patients, diagnostic labs, and payers. COVID-19, coronavirus disease 2019; HCP, healthcare professional.

through organizations such as the Genetic Alliance in the United States and United Kingdom[32,33]: increasing patient experience and skill sets will ensure better representation of the patient voice in current and future PM efforts.

The PM landscape is constantly and rapidly evolving.[11,34–45] Even over the past 2 years, the landscape has changed dramatically, with several important initiatives being introduced (e.g., Europe's Beating Cancer Plan,[46] EU Mission: Cancer,[47] No One Missed, by the US LUNGevity organization,[48] and Initiatives in Lung Cancer Care[49]). In addition, the US Cancer Moonshot was relaunched in February 2022, and this could be a driver for funding and innovation in PM. These initiatives bring opportunities for innovative new treatments, but this is only achievable within a healthcare system and governance structure that collectively have the agility to promote and adopt new developments; for example, the agility to develop multistakeholder, public sector, and private sector collaborations to deliver solutions.[50,51] Such collaborations can be termed an "intersectoral multistakeholder approach," because stakeholders from different sectors need to work together to integrate their strategies around PM. Efforts to provide PM across different stakeholder groups and regions highlight a shared awareness of its potential value (Figure 2).[40,42] We see this as a positive development that underscores the urgency to adapt our collective approach to PM.



01 Personalized Medicine Coalition
02 European Alliance for Personalised Medicine
03 International Consortium for Personalised Medicine
04 Swiss Personalized Health Network (SPHN)
05 Personalized Health and Related Technologies (PHRT)
06 Precision Medicine Alliance
07 Alliance for Precision Health
08 Global Alliance for Genomics and Health
09 European Personalised Medicine Association
10 Society for Functional Precision Medicine
11 American Association for Precision Medicine (AAMP)
12 Nordic Society of Human Genetics and Precision Medicine
13 European Alliance for Transformative Therapies
14 Nordic Trial Alliance
15 Nordic Health Research and Innovation Network
16 Nordic Alliance for Clinical Genomics
17 Nordic Biobank Network
18 Nordic Collaboration for Sensitive Data (Tryggve)
19 Network Genomic Medicine (NGM) Lung Cancer
20 Personalised medicine trials
21 EATRIS-Plus-Flagship in personalised medicine
22 Genomics England
23 European Society for Medical Oncology
24 APMI
25 National Comprehensive Cancer Network
26 Ontario Personalized Medicine Network
27 Genomic Medicine Sweden
28 The Cancer Collaborative
29 The European 1 million Genomes Initiative
30 National institute of health All of us research programme
31 Project Baseline
32 100,000 Genomes project
33 Estonian Personalised Medicine
34 China Precision Medicine Initiative
35 Taiwan Biobank
36 PMDI (Precision Medicine in Diabetes Initiative)
37 ACGP (Access to Comprehensive Genomic Profiling)
38 Precision Cancer Consortium PCC
39 CONNECT
40 Qatar Genome
41 Genomic Canada
42 Southern African Human Genome Programme (SAHGP)
43 Genome Netherlands
44 BigMed
45 GenomeAsia 100K
46 Brazilian Initiative on Precision Medicine
47 Melbourne Genomics Health Alliance (MGHA)
48 Global Genomic Medicine Collaborative
49 EU-STANDS4PM
50 The California Initiative To Advance Precision Medicine
51 EULAC-PerMed
52 WIN Consortium
53 Blood Profiling in Cancer (BIOODPAC) Consortium
54 Genomics Organization for Academic Laboratories
55 Canadian Personalized Healthcare Innovation Network (CPHIN)
56 OMICO
57 PREMIA, Precision Medicine Asia
58 EUCOPE
59 ALL CAN
60 Lungmapping Brazil

FIGURE 2 A diagram to illustrate the diversity of 60 precision medicine initiatives across the globe, categorized according to Policy, Research, Data sharing, Education, and Other.

Rapid increase in PM developments has led to a highly fragmented PM landscape, including ideas, goals, and issues to be solved, and this is multiplied by each stakeholder type. This can have unwarranted consequences for different stakeholder groups, including patients, whose care and outcomes can be affected (Box 1). In addition, access to PM is country dependent, with high variation in its availability (even within countries). The result is that individuals can have wildly different experiences of PM (Box 2). Fragmentation also means that attempts to expand PM are vertically siloed by scope or focus area, or by geography or disease type, meaning they are disconnected. This will continue without overarching leadership to bring these disparate—but complementary—efforts together.

BOX 1 The consequences of fragmentation: A patient perspective

With advances in research, we now understand some of the oncogene driver mutations and other alterations that occur in lung cancer. The development of therapies targeting these modifications has had a big impact within the community. However, many people who would benefit from PM approaches are not benefiting. The fragmented nature of the delivery of PM in lung cancer can lead to very different outcomes: it can mean the difference between life and death, and between poor and good quality of life.

Examples of the consequences of fragmentation:

- People whose tumors should have undergone testing, have not due to lack of testing availability, resulting in poorer outcomes due to substandard treatment options.
- People who have experienced a poor initial prognosis have had their outcome change significantly due to the identification of a targetable alteration and access to appropriate therapy.
- Testing is available but not the drug (and vice versa), resulting in poorer outcomes due to substandard treatment options.
- Poor communication of results so that people cannot reach out to appropriate oncogene-driven communities for support.
- Pressure being placed on patients having to know everything—which adds to stress rather than a need for awareness being stressed for both patients and HCPs.
- Pressure being placed on patients to take treatment quickly (as most are diagnosed with stage IV lung cancer) can be exacerbated by the long turnaround time for test results, and can impact future trial enrollment and access to targeted drugs (as some may be available only in a certain line setting).
- Many instances of people living with stable disease for many years—some now with no evidence of disease—because of access to appropriate therapy.
- Many believe they have been "lucky" to access testing, but it should not be about luck—it should be about best standard of care.

BOX 2 Patient experience of precision medicine is variable

Example: Variation in experience captured through a focus group of patients with cholangiocarcinoma diagnosed with gene mutation FGFR2 fusions, for which there is a targeted therapy.

One woman, whose clinician had been part of the clinical trials, was screened and offered the targeted therapy as a first treatment option rather than the third-line indication, sparing her surgery and chemotherapy. Another woman was forced to change physicians when hers refused to do the genomic testing, even when all previous interventions had failed; she did have the mutation and received the therapy but said she felt she had lost months of her life and quality of life. A third woman had started the targeted therapy but was afraid of having too much hope—precision medicine is not a cure.

Identifying and sharing best practices is key to achieving good outcomes. For example, providing genetic counselling (e.g., in a germline setting) and dedicated counsellors can help individuals understand and digest test results and their potential impact, and support shared decision-making.[52] However, fragmentation in PM makes it difficult to identify and share best practices, and there is little standardization across the PM pathway at national, international, and global levels. It is only by examining all these pieces and forging a logical, coordinated path that we can make sustainable changes to the healthcare system and stakeholder behaviors that will drive improved outcomes.

There is a need to better understand patients' experiences by gaining insight into what they feel, do, and need throughout the care pathway. This is particularly so for vulnerable individuals, for example, children, who require the involvement of a caregiver. There is limited understanding that patients' experiences encompass not only their symptoms and diagnosis, but also the frustrations, fears, anxieties, and preferences they have, and actions they take. We have discussed how important it is that the patient (or patient-advocate) voice is fully represented in PM. We believe that PM can only succeed through true patient-centric care and patient support programs, and that shared decision-making and implementation of shared decision-making tools are key. The value of a patient-centered approach has been demonstrated by patient-led groups successful in shaping and funding their own research agenda, by working directly with scientists, pharmaceutical and biotech companies, and other parties, to address unmet patient priorities.[53]

Taken together, the challenges for PM mean that many patients do not benefit from optimal treatment, and there is a health, societal, and economic impact. Today's healthcare system is not geared

toward the development and uptake of innovative medical interventions at scale; it is also failing to deliver the right care to many people.

3 | HOW CAN PM EVOLVE TO DELIVER BETTER PATIENT OUTCOMES?

We believe that balanced inclusion and collaboration between different stakeholders in PM, including patient communities, is necessary to reach the shared goal of making PM a reality for all those who could benefit from it. This requires increased awareness and education for patients/advocates and clinicians. It also needs a noncompetitive forum that connects all stakeholders (patients, patient organizations or advocates and caregivers, policymakers, regulators, healthcare professionals, pharmaceutical and diagnostics industries, health technology agencies) and builds positive momentum to make the shared goal of PM the priority. Patients must be equal partners and central to the PM approach—from early research through to clinical trials, approval of new treatments, and access to care—to ensure it represents their experiences and identifies barriers, solutions, and opportunities at the point of delivery. This approach ensures that outputs address unmet needs, have a real-world impact on the functional implementation of PM, and fosters a holistic view of PM with an emphasis on making the whole PM ecosystem work at each point along the care pathway. Given the variable status and availability/access to PM globally, and that PM is not appropriate for all diseases, it is also important to manage patient expectations of what is achievable and to not overpromise and underdeliver.

Collaboration is most needed across three pillars of activity: (1) data and evidence development to demonstrate the benefits of PM on patient-relevant outcomes; (2) education and tools for patients, clinicians, and other stakeholders to support informed decision-making and ultimately improve outcomes; and (3) addressing access barriers across the patient pathway to make PM an accessible reality for more people. These efforts would be supported by creating an international, intersectoral multistakeholder network to work on improved, sustainable solutions. While patient organizations and healthcare professionals acknowledge that PM could improve diagnostic efficiency and efficacious treatment, they express concerns over data privacy and potential misuse, ethics of prevention, access equity, and evidence of diagnostic accuracy and improved clinical outcomes with risks of overpromising and underperforming.[54–58]

A report outlining a framework to improve health technology assessment approaches to PM in oncology has highlighted the evolving landscape and the shift from conventional, large, randomized controlled trials toward basket trials and complex clinical trials. These types of trials and innovative ways of assessing PM may impact clinicians' confidence in PM approaches and their "belief" in the evidence for benefits of PM; this could make clinicians cautious of implementing PM approaches.[59] Unless clinician doubts are addressed and robust evidence is generated, adoption/availability of PM will be inadequate. Therefore, the knowledge gap between available data and clinicians' awareness/understanding of the data must be closed. It is important to acknowledge that many clinicians lack training in genomics or PM and will therefore be hesitant to adopt unfamiliar care approaches. One way to address this is by identifying training needs and collating and signposting to the existing resources providing practical guidance on the application of PM (in eligible patients), such as European Society for Medical Oncology recommendations for the use of next-generation sequencing in metastatic cancer,[60] the National Comprehensive Cancer Network Biomarkers Compendium, which aims to support decision-making on biomarker testing in people with cancer,[61] PM educational resources for healthcare professionals developed by the US Association of Community Cancer Centers (ACCC),[62] and the ACCC's process improvement toolkit for biomarker testing.[63] This training should also reflect insights from the patient's perspective, for example, on risk/benefit considerations, which can often be very different from the clinician's viewpoint. The ongoing COVID-19 pandemic has demonstrated that innovation can drive rapid improvements and developments through collaboration. This "shared-goal" approach could be applied to the PM agenda, incorporating the patient voice to understand what risks are acceptable and aligning the pace of change accordingly. Building a networked community of PM stakeholders would provide support and facilitate the implementation of PM. Application of existing PM guidelines with increased use of PM, and robust documentation of that use, will further build the evidence base for PM approaches.

We propose that contributors from all stakeholder groups should be involved in cocreating solutions to address issues within the three pillars of activity (education, evidence, and access), and a community of practice should be established with a common commitment to share learnings and establish best practices. This should be done in an environment that enables balanced input and decision-making across stakeholder groups, fosters trust, and facilitates open collaboration so that impact is achieved rapidly.

4 | A PRACTICAL ROADMAP FOR PM ADVANCEMENT

International coalitions provide a model for achieving intersectoral, multistakeholder synergy in PM. For example, Patient Focused Medicines Development (PFMD) was established in 2015 to embed meaningful patient engagement throughout the continuum of drug development. PFMD cocreated a meta-framework for patient engagement by mapping and connecting the fragmented landscape, and by learning from/building on best practices to develop guidance, tools, and resources for implementation of meaningful patient engagement.[64,65] This rational, stepwise approach can be translated to PM.

From Testing to Targeted Treatments (FT3), established in 2020, was created out of common experiences of success and frustration, but also a shared commitment to make PM the norm, rather than the exception. FT3 is a global, open, collaborative program with a

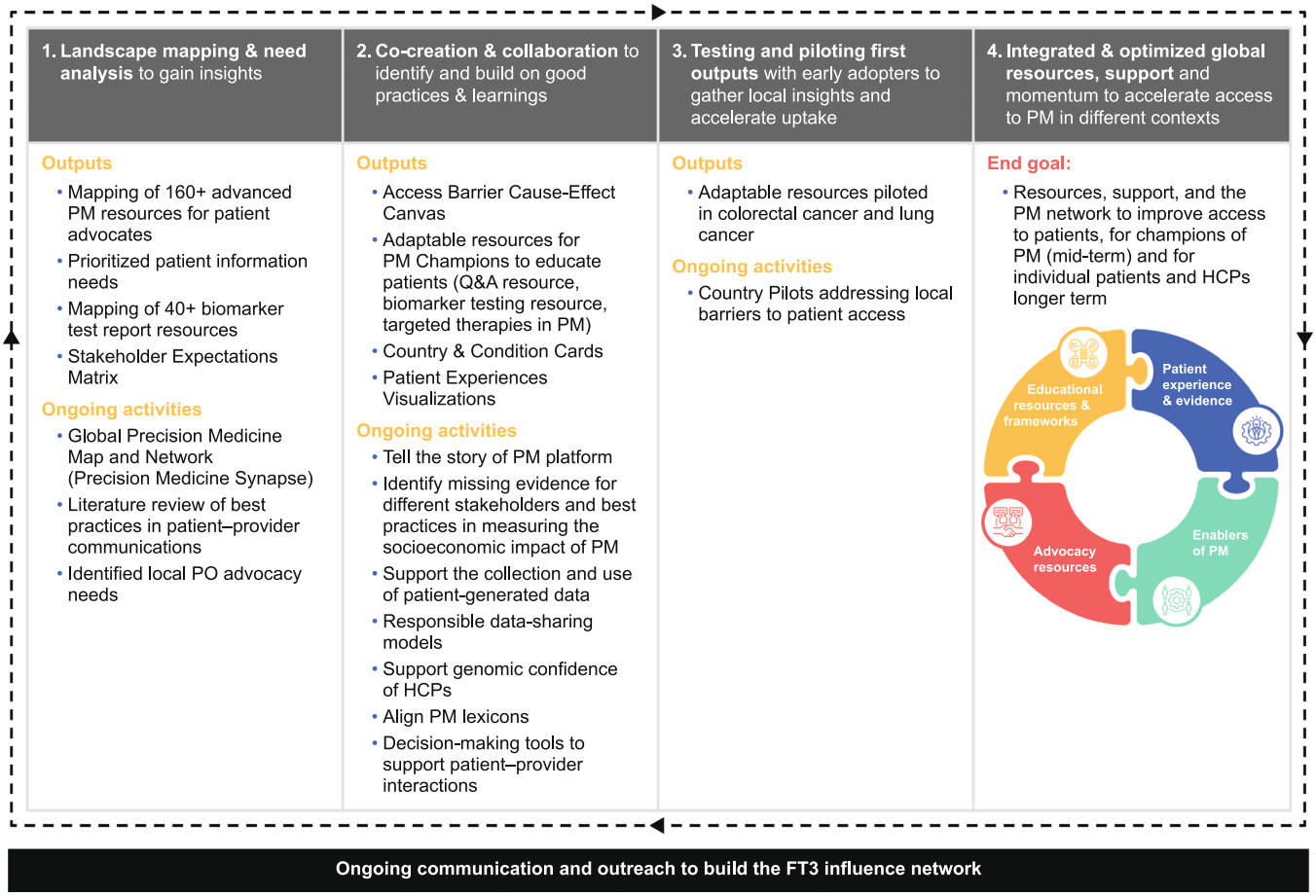


FIGURE 3 A diagram outlining a practical and iterative approach for precision medicine advancement in terms of output and ongoing activities, at the health system and healthcare delivery level. This begins with mapping the existing PM landscape and identifying unmet needs and priorities, to determine opportunities across geographic locations and conditions. Cocreation approaches will then be adopted to identify good practice and learning, followed by testing/piloting outputs to gather insights and accelerate uptake, leading to integrated and optimized global resources to accelerate PM. FT3, From Testing to Targeted Treatments; HCP, healthcare professional; PM, precision medicine; PO, patient organization.

collective goal to make PM a reality for all those who could benefit from it. FT3 unites diverse stakeholders including patients to identify synergies across disparate but complementary efforts, bringing them together to act as an incubator and accelerator for PM, starting with cancer and biomarker testing.

FT3 proposes a practical and iterative approach to advance PM at the health system level and healthcare delivery level. This begins with mapping the existing PM landscape and identifying common needs, gaps, priorities, and opportunities across geographic locations and conditions (Figure 3). Bringing together the broader ecosystem of individuals, initiatives, and organizations working in PM, and using examples and case studies to gain insights, will enable the identification and adoption of best practice approaches, thereby reducing duplication. Insights will also be captured from emerging models and "intermediate practice" that represent stepping stones along the PM journey: this pragmatic approach reflects global differences in PM "maturity" and acknowledges what is achievable in different situations. The initial aim of connecting and mapping the diverse PM landscape will require all stakeholders to share initiatives, experiences, and best practices. FT3 has developed The Global Precision Medicine Map and Network[66] to support the universal sharing of PM efforts (irrespective of scale or focus) and the discovery of active organizations, people, initiatives, and resources across the PM landscape. As of January 2023, over 773 organizations and 1062 resources have joined the map and network.

Where practices that have led to desired outcomes are lacking, FT3 is cocreating new solutions and driving efforts to fill the gaps, building on existing insights. The aim is to create an accessible repository of global tools and resources, covering the entire PM pathway, to build momentum for sustainably improving patient access to PM. These integrated and optimized tools can be customized to different disease contexts and applied across similar and/or appropriate healthcare systems, first through piloting and then sharing insights from pilots to scale up. A community of PM champions and early adopters (e.g., patient organizations, healthcare professional communities, and other healthcare system stakeholders) would support the development and dissemination of these resources. Although tools should be adapted to specific geographic

locations and diseases, the underlying understanding of access barriers, methodologies, learnings, best practices, and solutions can be shared globally and across diseases.

5 | A CALL TO ACTION FOR HEALTHCARE STAKEHOLDERS

PM provides an opportunity to transform lives. However, today's healthcare systems are not geared toward the development and uptake of innovative medical interventions at scale. This is due to many well-documented challenges, none of which can be overcome without balanced cocreation and collaboration involving all stakeholders. We need to understand the transformation required across stakeholder groups and collectively act to change the healthcare system if PM is to become a reality. For example, patient groups understand the opportunities PM can offer, and they can be educators/advocates for change, raising awareness of PM-driven approaches. In lung cancer, for example, evidence shows (and guidelines recommend) that biomarker testing is needed[60,61]; therefore, this should be built into the care pathway as standard. Here, stakeholders who are policymakers and decisionmakers should ensure that funding is available, while healthcare professionals should argue for the importance of biomarker testing and why it should be covered/reimbursed by healthcare systems. Governmental and institutional research ethics committees play an important role in approving clinical trials in PM, and they should consider the added value of innovative trials in therapeutic areas where approved treatments are lacking. Finally, to change mindsets and to fully embed PM in future treatment pathways, it will be important to integrate PM within the medical curriculum globally, to enable early preparation of future HCPs.

The rapid changes in the PM landscape underscore the need for a dynamic approach so that changes can be made quickly within healthcare delivery, to ensure those who can benefit from existing (and rapidly emerging) approaches are able to derive benefit. Guidelines consider data and evidence that are available during guideline development. Given the fast-changing landscape, guidelines should also consider emerging strategies (e.g., being assessed in clinical trials) and not only approved medications, so that when new agents become available, guidance is not far behind. PM approaches are often seen as a "luxury" and outside of conventional treatments, but it is no longer a new concept and should be an accepted diagnostic/treatment option where appropriate and available. It is also important to acknowledge that PM is not currently an accessible reality for the majority.[67,68] To enable patient-centric care and sustainably improve patient experiences and outcomes, the patient community must be closely involved in making decisions and designing solutions. A joined-up and stepwise approach to delivering PM can be achieved through improving education and awareness for patients and healthcare professionals, conducting biomarker-driven trials, and implementing tumor testing as standard with corresponding drugs and other needed support available. By working together, we will make PM an accessible reality for everyone who could benefit from it.

6 | CONCLUSIONS

Shared opportunities to advance PM can only be achieved through multistakeholder and intersectoral collaboration, working with patients to implement accessible and sustainable PM approaches. We propose a practical roadmap for PM that begins with uniting diverse stakeholders to connect the fragmented PM landscape and to amplify and augment existing best practices. Cocreation efforts would focus on closing gaps where practices are lacking and developing a user-friendly, translatable suite of resources to support the delivery of PM approaches that can transform lives.

AUTHOR CONTRIBUTIONS

Anne-Marie Baird: Conceptualization; writing—review and editing. **C. Benedikt Westphalen**: Conceptualization; writing—review and editing. **Sandra Blum**: Conceptualization; writing—review and editing. **Begonya Nafria**: Conceptualization; writing—review and editing. **Tanya Knott**: Conceptualization; writing—review and editing. **Ify Sargeant**: Writing—original draft; writing—review and editing. **Helena Harnik**: Conceptualization; writing—review and editing. **Nicholas Brooke**: Conceptualization; writing—review and editing. **Nicole Wicki**: Conceptualization; writing—review and editing. **Durhane Wong-Rieger**: Conceptualization; writing—review and editing.

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CONFLICTS OF INTEREST STATEMENT

Sandra Blum is an employee of Roche, Basel, Switzerland. Tanya Knott reports consulting fees from Rare Revolution, support for meeting attendance/travel from Roche Products Ireland ESMO Sponsorship, and Roche Sponsorship for hosting World CUP Awareness Week. Sandra Blum, Tanya Knott, C. Benedikt Westphalen, Begonya Nafria, and Durhane Wong-Rieger hold a leadership or fiduciary role in the "From Testing to Targeted Treatments" (FT3) Board. Helena Harnik, Nicholas Brooke and Nicole Wicki are collaborating with The Synergist, a nonprofit organization, whose

programs, including the FT3 program, are sponsored by the industry. However, the authors declare that these supporting funding sources/financial relationships and additional roles did not provide any conflict of interest with this project. All other authors declare that they have no conflicts of interest. The views and opinions of the authors here do not reflect the opinions of their respective organizations; they reflect personal accounts from the varied expertise and perspectives. All authors are members of FT3, and all had a role in the design of the project, review, and interpretation of PM initiatives identified, including PM case studies; writing/reviewing of the report; and the decision to submit the report for publication.

DATA AVAILABILITY STATEMENT

Data sharing is not applicable to this article as no new data were created or analyzed in this study.

TRANSPARENCY STATEMENT

The lead author Helena Harnik affirms that this manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted; and that any discrepancies from the study as planned (and, if relevant, registered) have been explained.

ORCID

Helena Harnik http://orcid.org/0000-0001-6690-1459

REFERENCES

1. MedlinePlus. *What is Precision Medicine?* MedlinePlus; 2021.
2. European Commission. *Definition of Personalised Medicine*. European Commission; 2021.
3. Wilsdon T, Barron A, Edwards G, et al. *The Benefits of Personalised Medicine to Patients, Society and Healthcare Systems*. Charles River Association; 2018:1-72.
4. Personalized Medicine Coalition. *Moving Beyond Population Averages. A Patient-Centered Research Agenda Advancing Personalized Medicine*. PMC; 2020.
5. Peck RW. The right dose for every patient: a key step for precision medicine. *Nat Rev Drug Discov*. 2016;15:145-146.
6. Nassar SF, Raddassi K, Ubhi B, Doktorski J, Abulaban A. Precision medicine: steps along the road to combat human cancer. *Cells*. 2020;9:2056.
7. Seyhan AA, Carini C. Are innovation and new technologies in precision medicine paving a new era in patients centric care? *J Transl Med*. 2019;17:114.
8. van der Velden DL, Hoes LR, Van Der Wijngaart H, et al. The drug rediscovery protocol facilitates the expanded use of existing anticancer drugs. *Nature*. 2019;574:127-131.
9. La Russa R, Finesch V, Di Sanzo M, et al. Personalized medicine and adverse drug reactions: the experience of an Italian teaching hospital. *Curr Pharm Biotechnol*. 2017;18:274-281.
10. Parimbelli E, Marini S, Sacchi L, Bellazzi R. Patient similarity for precision medicine: a systematic review. *J Biomed Inf*. 2018;83:87-96.
11. Personalized Medicine Coalition. *The Personalized Medicine Report 2020. Opportunity, Challenges, and the Future*. PMC; 2020.
12. European Alliance for Personalised Medicine. *From Here to 2025: Personalised Medicine and Healthcare for an Immediate Future*. European Alliance for Personalised Medicine; 2017.
13. Rebuzzi SE, Zullo L, Rossi G, et al. Novel emerging molecular targets in non-small cell lung cancer. *Int J Mol Sci*. 2021;22:2625.
14. Tafe LJ. Non-small cell lung cancer as a precision oncology paradigm: emerging targets and tumor mutational burden (TMB). *Adv Anat Pathol*. 2020;27:3-10.
15. Grant MJ, Herbst RS, Goldberg SB. Selecting the optimal immunotherapy regimen in driver-negative metastatic NSCLC. *Nat Rev Clin Oncol*. 2021;18:625-644.
16. Dupont CA, Riegel K, Pompaiah M, Juhl H, Rajalingam K. Druggable genome and precision medicine in cancer: current challenges. *FEBS J*. 2021;288:6142-6158.
17. Ahmed Z, Mohamed K, Zeeshan S, Dong X. Artificial intelligence with multi-functional machine learning platform development for better healthcare and precision medicine. *Database*. 2020;2020:baaa010.
18. Soto F, Wang J, Ahmed R, et al. Medical micro/nanorobots in precision medicine. *Adv Sci*. 2020;7:2002203.
19. Bertier G, Carrot-Zhang J, Ragoussis V, Joly Y. Integrating precision cancer medicine into healthcare—policy, practice, and research challenges. *Genome Med*. 2016;8:108.
20. International Quality Network for Pathology (IQNPath), European Cancer Patient Coalition; European Federation of Pharmaceutical Industries and Associations (EFPIA). *Unlocking the Potential of Precision Medicine in Europe. Improving Cancer Care Through Broader Access to Quality Biomarker Testing. Policy Recommendations*. IQNPath and EFPIA; 2021.
21. Hamazaki T, Okuyama H, Ogushi Y, Hama R. Towards a paradigm shift in cholesterol treatment. A re-examination of the cholesterol issue in Japan. *Ann Nutr Metab*. 2015;66(suppl 4):1-116.
22. Chung WK, Erion K, Florez JC, et al. Precision medicine in diabetes: a consensus report from the American Diabetes Association (ADA) and the European Association for the Study of Diabetes (EASD). *Diabetologia*. 2020;63:1671-1693.
23. Hampel H, Vergallo A, Perry G, Lista S. The Alzheimer precision medicine initiative. *J Alzheimer's Dis*. 2019;68:1-24.
24. Tebani A, Afonso C, Marret S, Bekri S. Omics-based strategies in precision medicine: toward a paradigm shift in inborn errors of metabolism investigations. *Int J Mol Sci*. 2016;17:1555.
25. Chaytow H, Faller KME, Huang YT, Gillingwater TH. Spinal muscular atrophy: from approved therapies to future therapeutic targets for personalized medicine. *Cell Rep Med*. 2021;2:100346.
26. Spadarella G, Di Minno A, Milan G, et al. Paradigm shift for the treatment of hereditary haemophilia: towards precision medicine. *Blood Rev*. 2020;39:100618.
27. Harutyunyan M, Huang Y, Mun KS, Yang F, Arora K, Naren AP. Personalized medicine in CF: from modulator development to therapy for cystic fibrosis patients with rare CFTR mutations. *Am J Physiol Lung Cell Mol Physiol*. 2018;314:L529-L543.
28. Marcon AR, Bieber M, Caulfield T. Representing a "revolution": how the popular press has portrayed personalized medicine. *Genet Med*. 2018;20:950-956.
29. LUNGevity Foundation. LUNGevity; 2023. https://www.lungevity.org
30. Lung Cancer Europe (LuCE). *IV LuCE Report on Lung Cancer: Early Diagnosis and Screening Challenges In Lung Cancer*. LuCE; 2019.
31. Dolgin E. Oncogene-specific advocacy groups bring a patient-centric perspective to studies of lung cancer. *Nature*. 2020;587:S16-S17.
32. Genetic Alliance. *Genetic Alliance Through the Years*. Genetic Alliance; 2023.
33. pharmaphorum. *Building a Better Future for People With Rare Diseases in All Four UK Nations*. pharmaphorum.
34. Li A, Bergan RC. Clinical trial design: past, present, and future in the context of big data and precision medicine. *Cancer*. 2020;126:4838-4846.

35. Hulsen T, Jamuar SS, Moody AR, et al. From big data to precision medicine. *Front Med*. 2019;6:34.
36. Patel M, Kato SM, Kurzrock R. Molecular tumor boards: realizing precision oncology therapy. *Clin Pharm Ther*. 2018;103:206-209.
37. Love-Koh J, Peel A, Rejon-Parrilla JC, et al. The future of precision medicine: potential impacts for health technology assessment. *Pharmacoeconomics*. 2018;36:1439-1451; Erratum in: Pharmacoeconomics. 2019;37:117.
38. Faulkner E, Holtorf AP, Walton S, et al. Being precise about precision medicine: what should value frameworks incorporate to address precision medicine? A report of the personalized precision medicine special interest group. *Value Health*. 2020;23:529-539; Erratum in: Value Health. 2020;23:1522.
39. Ginsburg GS, Phillips KA. Precision medicine: from science to value. *Health Aff*. 2018;37:694-701.
40. Cirillo D, Valencia A. Big data analytics for personalized medicine. *Curr Opin Biotechnol*. 2019;58:161-167.
41. Stark Z, Dolman L, Manolio TA, et al. Integrating genomics into healthcare: a global responsibility. *Am J Hum Genet*. 2019;104:13-20.
42. Lee J, Hamideh D, Nebeker C. Qualifying and quantifying the precision medicine rhetoric. *BMC Genomics*. 2019;20:868.
43. National Academies of Sciences, Engineering, and Medicine (NASEM). Advancing disease modeling in animal-based research in support of precision medicine. In: *Proceedings of a Workshop. 2, Existing Precision Medicine Initiatives*. National Academies Press; 2018. doi:10.17226/25002
44. Facts and Factors. *Europe Precision Medicine Market by Technology (Big Data Analytics, Gene Sequencing, Companion Diagnostics, Bioinformatics, Drug Discovery, and Others), by End-user (Diagnostic Tool Companies, Clinical Labs, Healthcare IT/Big Data Companies, and Pharma & Biotech Companies), and by Application (Oncology, Respiratory, CNS, Immunology, and Others): Industry Perspective, Comprehensive Analysis, and Forecast, 2018–2025*. Facts and Factors; 2023.
45. ReportLinker. *Global Precision Medicine Market: Focus on Precision Medicine Ecosystem, Applications, 13 Countries Mapping, and Competitive Landscape—Analysis and Forecast: 2018-2028*. BIS Research; 2023.
46. European Commission. *Europe's Beating Cancer Plan*. European Commission; 2023.
47. European Commission. *EU Mission: Cancer*. European Commission; 2023.
48. LUNGevity Foundation. *No One Missed*. LUNGevity Foundation; 2023.
49. The Lung Ambition Alliance. *Initiatives in Lung Cancer Care (ILC2)*. The Lung Ambition Alliance; 2023.
50. World Economic Forum. *Briefing Paper: Agile Governance for Creative Economy 4.0*. World Economic Forum; 2019.
51. Brookings Institute. *Advancing Precision Medicine Through Agile Governance: Bridging Innovation and Regulation for the Greater Good*. Brookings Institute; 2023.
52. Amendola LM, Golden-Grant K, Scollon S. Scaling genetic counseling in the genomics era. *Annu Rev Genomics Hum Genet*. 2021;22:339-355.
53. Goldman L. PL02.06 patient-directed innovation, research and clinical trials. *J Thorac Oncol*. 2021;16:S64-S65.
54. Erdmann A, Rehmann-Sutter C, Bozzaro C. Patients' and professionals' views related to ethical issues in precision medicine: a mixed research synthesis. *BMC Med Ethics*. 2021;22:116.
55. Schleidgen S, Marckmann G. Re-focusing the ethical discourse on personalized medicine: a qualitative interview study with stakeholders in the German healthcare system. *BMC Med Ethics*. 2013;14:20.
56. Beans JA, Woodbury RB, Wark KA, Hiratsuka VY, Spicer P. Perspectives on precision medicine in a tribally managed primary care setting. *AJOB Empir Bioeth*. 2020;11:246-256.
57. Cho HN, Shin SY, Hwangbo B, et al. Views on precision medicine among health professionals in Korea: a mixed methods study. *Yonsei Med J*. 2020;61:192-197.
58. Rauter CM, Wöhlke S, Schicktanz S. My data, my choice? German patient organizations' attitudes towards big data-driven approaches in personalized medicine. An empirical–ethical study. *J Med Syst*. 2021;45:43.
59. Shift Health. *When Precision Meets Decision*. Shift Health; 2023.
60. Mosele F, Remon J, Mateo J, et al. Recommendations for the use of next-generation sequencing (NGS) for patients with metastatic cancers: a report from the ESMO Precision Medicine Working Group. *Ann Oncol*. 2020;31:1491-1505.
61. National Comprehensive Cancer Network (NCCN). *Biomarkers Compendium*. NCCN; 2023.
62. Association of Community Cancer Centers (ACCC). *Precision Medicine*. ACCC; 2023.
63. Association of Community Cancer Centers (ACCC). *BiomarkerLIVE Toolkit*. ACCC; 2023.
64. Hoos A, Anderson J, Boutin M, et al. Partnering with patients in the development and lifecycle of medicines: a call for action. *Ther Innov Regul Sci*. 2015;49:929-939.
65. Deane K, Delbecque L, Gorbenko O, et al. Co-creation of patient engagement quality guidance for medicines development: an international multistakeholder initiative. *BMJ Innovations*. 2019;5:43-55.
66. Precision Medicine Synapse. *The Global Precision Medicine Map and Network*. Precision Medicine Synapse; 2023.
67. Horton S, Sullivan R, Flanigan J, et al. Delivering modern, high-quality, affordable pathology and laboratory medicine to low-income and middle-income countries: a call to action. *Lancet*. 2018;391:1953-1964.
68. Rehman A, Awais M, Baloch NUA. Precision medicine and low- to middle-income countries. *JAMA Oncol*. 2016;2:293-294.

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