

Personalized Medicine Legislation **Legislative Specifications**

Concept List

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6. Ensuring Patient Access to Personalized Medicine Through Delivery System Reforms
 - (A) Center for Medicare and Medicaid Innovation

Legislative Findings:

Congress finds that –

- (1) Progress toward the development and adoption of personalized medicine depends on continuing the shared commitment to collaborative research efforts undertaken to date by various public- and private-sector shareholders.
- (2) Given the substantial costs of developing biomedical advances, companies may be unable or unwilling to invest in promising lines of research due to uncertainties regarding costs and return on investment without a streamlined regulatory environment together with certain incentives.
- (3) The current and evolving regulatory environment for personalized medicine increases the cost of innovating in the area of personalized medicine. Federal incentives, including a coordinated and expedited clearance and approval process for personalized medicine drugs/biologics as well as personalized medicine diagnostics, where applicable, whether developed independently or together, can reduce this burden and increase the attractiveness of developing these potentially lifesaving technologies.
- (4) The costs of co-developing personalized medicine drugs/biologics as well as personalized medicine diagnostics, including the conduct of appropriate clinical trials and payment of patent royalties, are particularly substantial. Federal grants and tax credits are necessary to encourage the rapid development and marketing of these potentially life saving technologies, and in order to facilitate patient access.
- (5) The lack of coordinated coverage of personalized medicine drugs/biologics and personalized medicine diagnostics by Medicare and other third party payors threatens patient access to these promising technologies. Ensuring such coverage, when medically necessary, will help facilitate patient access. However, these improvements in coverage must be achieved without adversely affecting patient access to other valuable healthcare services.
- (6) Recent legislation established programs that aim to improve the quality of care while reducing costs through the development of new health care delivery models. Incorporating incentives to appropriately use personalized medicine into these programs will further Congress' goals of reducing costs and improving health care quality.

CONCEPT 1: Ensuring Adequate Representation of Personalized Medicine Perspective Through Advisory Committees

Health and Human Services Personalized Medicine Advisory Committee: A multitude of policies and regulations adopted by the Department of Health and Human Services (HHS) have implications for personalized medicine, including the FDA approval process, coverage and reimbursement under publicly funded health care programs (e.g., Medicare and Medicaid), policies regarding the training and retention of the U.S. healthcare workforce, newborn and other screening programs, and policies as to which research is to be conducted by federal agencies. However, there is currently no advisory body within HHS that ensures that such policies, procedures, regulations, and programs recognize the unique challenges in the area of personalized medicine, or that the personalized medicine perspective is adequately represented on existing and future HHS advisory bodies. This section would amend the Public Health Service Act to establish a Personalized Medicine Advisory Committee (“Committee”) within the Office of the Secretary to support the advancement of personalized medicine within HHS. The Committee would be comprised of stakeholders from both the public and the private sector. Specifically, the Committee would be comprised of:

- Representatives appointed by the Secretary of HHS from the following agencies:
 - The Food and Drug Administration;
 - The National Institutes of Health;
 - The Centers for Medicare & Medicaid Services;
 - The Centers for Disease Control and Prevention; and
 - The Office of the National Coordinator for Health Information Technology.
- Representatives appointed by the Comptroller General from the private sector, including:
 - Two academic or independent researchers involved in research related to personalized medicine;
 - Two representatives from pharmaceutical or biotechnology companies involved in the research and development of personalized medicine drugs/biologics;
 - Two representatives of medical device and diagnostics companies involved in the research and development of personalized medicine diagnostics;
 - Two representatives of clinical laboratories, including a laboratory medical director;
 - Two practicing physician representatives, including at least one physician with expertise in the area of personalized medicine;
 - Two patient representatives;
 - Two representatives of healthcare providers, such as hospitals and genetic counselors, that offer services related to personalized medicine;
 - One representative from a quality improvement organization; and
 - One representative with expertise in the area of health information technology (HIT).

[Note to Congressional Legislative Counsel: Consider designating a primary/secondary representative from each of the categories with two members]

The specific roles of the Committee would be to:

- Review and make recommendations regarding the policies, procedures, regulations, and programs of HHS to ensure that they both: (1) Foster further publicly- and privately-funded research and innovation in the area of personalized medicine; and (2) Promote timely and appropriate patient access to personalized medicine drugs/biologics, personalized medicine diagnostics, and related items and services, including services rendered by physicians and genetic counselors, under federally funded healthcare programs.
- In conducting this review, the Committee would be required to prioritize the review of the HHS policies, procedures, regulations, and programs related to:
 - The development, approval, and marketing of personalized medicine drugs/biologics and personalized medicine diagnostics;
 - The conduct of Comparative Effectiveness Research (CER) and health technology assessments;
 - Coverage and reimbursement under Medicare, Medicaid, and SCHIP;
 - Benefit design and cost-sharing under both public and private healthcare programs;
 - Coordination of policies and regulations within HHS and its related Agencies;
 - The conduct of programs that aim to improve the delivery and outcomes of care; and
 - The training and retention of the U.S. healthcare workforce.

Additional duties of the Committee include to:

- Submit a report to Congress, within 18 months of enactment and annually thereafter, regarding: the activities of the committee; any identified scientific, regulatory, reimbursement, or other barriers to personalized medicine research and access to personalized medicine drugs/biologics and personalized medicine diagnostics identified by the committee; and policy and legislative recommendations to address these barriers.
- Review the membership of existing advisory committees within HHS and make recommendations to the Secretary as to whether there is a need for greater participation by individuals with expertise in the area of personalized medicine.

This section would also set forth details regarding the terms, appointments, chairperson/vice-chairperson, compensation, and meetings of the Committee. It would appropriate funding for fiscal years 2012 through 2019.

Medicare Payment Advisory Commission: The Medicare Payment Advisory Commission (MedPAC) is an independent Congressional agency established by the Balanced Budget Act of 1997 to advise Congress on issues affecting the Medicare program. Existing law sets forth certain criteria with respect to individuals eligible to serve on the commission. This section would amend the law to require that MedPAC include individuals with expertise in the area of personalized medicine.

National Healthcare Workforce Commission: PPACA established the National Healthcare Workforce Commission (“Commission”) to advise the President, Congress, federal agencies, and

states regarding issues related to the healthcare workforce. Among other things, the Commission is required to review the health care workforce needs of special populations, such as minorities, rural populations, gender-specific needs, individuals with disabilities, and others. This section would amend the list of special populations addressed by this review to include individuals with different treatment responses that result from genetic characteristics.

Relevant Definitions: This section would amend the Public Health Service Act to include the following definitions:

Personalized Medicine: The term “personalized medicine” means the use of an individual’s family history, current health status, environmental determinants of health, genomically-derived information, or molecular data to better target the delivery of health care.

Personalized Medicine Drug/Biologic: Cross-reference definition under Social Security Act (Concept 3).

Personalized Medicine Diagnostic: Cross-reference definition under Social Security Act (Concept 3).

CONCEPT 2: Incentivize Personalized Medicine by Creating a Transparent and Predictable Regulatory Environment for Personalized Medicine Products

Coordinated review of personalized medicine products. Under current law, FDA is generally not required to coordinate its review of medical devices and drugs/biologics, including related personalized medicine products that concurrently undergo review by two different Centers within the FDA. Thus, there is a concern that a qualified targeted drug/biologic may be approved before a related qualified companion diagnostic receives approval, effectively delaying the use of that drug or biologic, and vice versa. This section would establish an Office of Personalized Medicine within the Office of the Commissioner at FDA to ensure the clear, efficient, and integrated premarket review of personalized medicine products, including a qualified targeted drug/biologic or a qualified companion diagnostic. This Office would be required to employ experts with knowledge in both the drug/biologic and device regulatory processes. The Office of Personalized Medicine would have the authority to:

- Ensure that the reviews of personalized medicine products, including a qualified targeted drug/biologic and a qualified companion diagnostic, are conducted in a coordinated manner;
- Ensure that personalized medicine products, including a qualified targeted drug/biologic and a qualified companion diagnostic, are being reviewed in accordance with targeted timelines;
- Review FDA's guidance, policies, procedures, and practices, as well as the agency's intercenter agreements that relate to personalized medicine products, and make recommendations to ensure that they facilitate the review and consistent regulation of such products;
- Be involved in dispute resolution pertaining to the review of personalized medicine products, including a qualified targeted drug/biologic or a qualified companion diagnostic;
- Make presentations to FDA Advisory Committees, as appropriate; and
- Study and compile data on the activity of the FDA Centers that review personalized medicine products to determine whether the goals of this Office are being met.

The FDA would be required to report to Congress on the activities and impact of the Office of Personalized Medicine and the Centers within FDA that review personalized medicine products, including the timeliness of the FDA's review of such products.

Concurrent Review of qualified companion diagnostics. Under current law, the timing of the FDA's review of a drug/biologic is unrelated to the timing of the agency's review of a device. As a result, a drug/biologic may be available on the market prior to the approval of a device that is prescribed, recommended, or suggested for purposes of identifying subgroups for which the drug/biologic should be used, or to determine the safe and effective dosing or use of the product. This section would amend the Federal Food, Drug, and Cosmetic Act, which would require FDA to:

- Review and act on an application for a product intended to be a qualified companion diagnostic within 30 days after the date the application for the product intended to be a related qualified targeted drug/biologic is approved or licensed, as long as the FDA receives the application for the product intended to be a qualified companion diagnostic before or at the same time as the application for the product intended to be a qualified targeted drug/biologic. The review of the application for the product intended to be a qualified companion diagnostic may not exceed 180 days;
- Review and act on an application for a product intended to be a qualified companion diagnostic that is received within 60 days after the date on which the FDA issues an order approving the application for a related product intended to be qualified targeted drug/biologic

no later than 60 days after the date on which the application for the product intended to be a qualified companion diagnostic is received. The review of the application for the product intended to be a qualified companion diagnostic may not exceed 180 days;

- Review and act on other applications for products intended to be qualified companion diagnostics within 180 days of receipt of any such application (a “180 day review”) for premarket approval submissions; and
- Coordinate the consistent review of the diagnostic and the drug/biologic, taking care to limit any overlap of review activities.

Relevant definitions: This section would amend the Federal Food, Drug, and Cosmetic Act to add the following definitions:

Personalized Medicine Products: Means two or more drugs, biologics, or devices that are prescribed, recommended, referenced, or suggested in the labeling for one or more such products, as being intended to be used together for purposes of tailoring treatment to the genetic or genomic characteristics of a subpopulation of patients, or other characteristics deemed appropriate by the Secretary. Such term shall include both a qualified targeted drug/biologic and a qualified companion diagnostic.

Qualified Targeted Drug/Biologic: For purposes of sections [##] of this Act, the term “Qualified Targeted Drug/Biologic” means a product approved by the Secretary under section 505 of this Act, or licensed by the Secretary under section 351 of the Public Health Service Act, for which a qualified companion diagnostic is prescribed, recommended, referenced, or suggested in its labeling, or in the labeling of the qualified companion diagnostic, for purposes of—

- (1) identifying selected subgroups of a larger population with a disease or condition for whom use of the product is considered safe and effective based on the patients’ genetic and genomic characteristics, or other characteristics deemed appropriate by the Secretary; or
- (2) determining the safe and effective dosing or use of the product.

Qualified Companion Diagnostic: For purposes of sections [##] of this Act, the term “Qualified Companion Diagnostic” means a product cleared, approved, or licensed by the Secretary under sections 510(k), 515, or 505 of this Act, or section 351 of the Public Health Service Act, for use in conjunction with a qualified targeted drug/biologic, or other product or products identified by the Secretary, to inform the selection, initiation, dosing, or monitoring, or avoidance of treatment with such product or products.

[Note to Congressional Legislative Counsel: Insert section numbers of the FFDCFA that correspond to the “Coordinated Review of Personalized Medicine Products” and “Concurrent Review of Qualified Companion Diagnostics” in place of [##]]

[Note: PMC is currently still working to obtain full support for the definition of “Qualified Targeted Drug/Biologic” from all PMC members]

Rule of Statutory Construction: All amendments to the Federal Food, Drug, and Cosmetic Act

made by this section apply with equal force to a product licensed under section 351 of the Public Health Service Act.

CONCEPT 3: Medicare Coverage of Personalized Medicine Diagnostics and Related Items and Services

Medicare Coverage of Personalized Medicine Diagnostics: Under current law, whether Medicare provides coverage for a particular drug/biologic is independent of whether it provides coverage for a particular diagnostic, and vice versa. This section would amend the Medicare statute to provide statutory coverage of a personalized medicine diagnostic that is prescribed, recommended, referenced, or suggested for use in the FDA-approved labeling of a personalized medicine drug/biologic for which Medicare coverage is available. This coverage would not be contingent on the issuance of a national coverage determination (NCD) or local coverage determination (LCD).

Medicare Coverage of Genetic Condition Diagnostic Tests: The Medicare program only covers and reimburses items that are “reasonable and necessary”. Under this standard, a Medicare beneficiary must generally show signs or symptoms of the condition before Medicare will cover diagnostic tests. Therefore, diagnostic tests, including genetic tests, are generally not covered solely on the basis of an individual’s family history or exposure to environmental hazards. This section would amend the Medicare statute to provide statutory coverage of “genetic condition diagnostic tests” – defined to include multi-gene tests – when an individual shows any signs or symptoms associated with a genetic condition or cancer, or on the basis of the individual’s family history of a genetic condition or cancer or exposure to environmental health conditions likely to result in a genetic condition or cancer. This coverage would be extended without the need for an NCD or LCD. This section would also reimburse such services under the physician fee schedule or the clinical laboratory fee schedule, as appropriate.

Relevant Definitions: This section would amend the Social Security Act in section 1861(s)(2) to include the following definitions:

Personalized Medicine Diagnostic: Means an item or service used in conjunction with a personalized medicine drug/biologic, or other product or products identified by the Secretary, to inform the selection, initiation, dosing, or monitoring of, or avoidance of treatment with such product or products.

Personalized Medicine Drug/Biologic: Means a drug or biologic that is tailored to the genetic or genomic characteristics of an individual patient or subpopulation of patients, or other characteristics deemed appropriate by the Secretary.

This section would also amend the Social Security Act to add “genetic condition diagnostic test” to the definition of “medical and other health services” under section 1861(s)(2), and would add the following, more detailed definition of this term at the end of section 1861:

Genetic Condition Diagnostic Test

“(a)(1) The term ‘genetic condition diagnostic test’ means a test or tests furnished to

an individual at risk for or diagnosed with a genetic condition or cancer (as defined in paragraph (2)) that analyzes human DNA, RNA, genes, chromosomes, and/or genomes to detect heritable genotypes, phenotypes, karyotypes, or mutations that cause or are likely to cause a specific disease or condition, or that analyzes human proteins and certain metabolites, which are used to detect heritable genotypes, phenotypes, or mutations in order to predict or assess an individual's risk of disease for purposes of directing clinical management, including to direct the use of personalized medicine drugs/biologics. The term 'genetic condition diagnostic test' includes, but is not limited to, a test capable of testing one or more such analytes and comprehensive genomic tests.

“(2) For purposes of paragraph (1), the term ‘individual at risk for or diagnosed with a genetic condition or cancer’ means an individual who:

“(i) shows any signs or symptoms associated with the genetic condition or cancer;

“(ii) has a family history of the genetic condition or cancer; or

“(iii) has been exposed to environmental health conditions likely to result in the genetic condition or cancer.”

CONCEPT 4: Expedited Exceptions Process for a Personalized Medicine Drug/Biologic Under Medicare Part D

Under federal law, Medicare Part D plan sponsors are required to operate an exceptions and appeals process that provides enrollees with the opportunity to challenge the exclusion of a particular drug from a plan's formulary or the placement of a drug on a higher cost-sharing tier. This section would amend the Medicare statute to require that a request for an exception that involves a personalized medicine drug/biologic be made on an expedited basis (i.e., 24 hours within receiving materials from the enrollee).

Relevant Definitions: Cross-reference definition of a personalized medicine drug/diagnostic in the Social Security Act (Concept 3).

Concept 5: Personalized Medicine Research and Development Tax Credits

Expand and extend the QTDP established by PPACA. The Patient Protection and Affordable Care Act (PPACA) amended the Internal Revenue Code to establish a Qualifying Therapeutic Discovery Tax Project (QTDP) tax credit/cash grant program to provide smaller life sciences companies that would generally not be able to benefit from tax credits with an opportunity to recover the cost of certain spending and investments. The amount of the credit is equal to 50 percent of eligible expenses incurred in 2009 or 2010 on a project that aims to: treat an unmet medical need, or prevent, detect or treat chronic or acute diseases and conditions; reduce long-term healthcare costs; or significantly advance the goal of curing cancer. This section would amend the Internal Revenue Code to extend the availability of QTDP grants and credits through 2017 and would expand the definition of projects eligible for such grants and credits to also include projects that aim to further the development of a personalized medicine drug/biologic or a personalized medicine diagnostic.

Personalized medicine research and development credits. The costs of developing a personalized medicine drug/biologic or a personalized medicine diagnostic, including the conduct of appropriate clinical trials and payment of patent royalties, are particularly substantial, and there is little incentive for pharmaceutical, diagnostic kit, and laboratory companies to work together to co-develop them. In addition, challenges remain for companies that have been encouraged by HHS (including FDA) to develop a personalized medicine drug/biologic or a personalized medicine diagnostic and would like to do so. Federal grants and tax credits are necessary to encourage the rapid development and marketing of these potentially lifesaving technologies and to enhance the competitiveness of the United States in research and development in this area. This section would amend the Internal Revenue Code to establish a research and development tax credit to cover the costs of qualified research incurred by entities that either:

- Have entered into a formal agreement with an entity that is developing or has received licensure or approval of a personalized medicine drug/biologic for purposes of developing a personalized medicine diagnostic that will support the safety, efficacy, or responsiveness of such personalized medicine drug/biologic;
- Have entered into a formal agreement with an entity that is developing or has received licensure or approval of a personalized medicine diagnostic for purposes of creating a product intended for approval as a personalized medicine drug/biologic that is tailored to a sub-population of patients identified by the personalized medicine diagnostic;
- Have been requested by the Secretary of HHS to develop a personalized medicine diagnostic or a personalized medicine drug/biologic, or both.
- Are developing a personalized medicine drug/biologic or a personalized medicine diagnostic that either:
 - The Secretary of HHS determines will:
 - Address an unmet medical need;
 - Substantially improve the utility of one or more therapeutic or prophylactic compounds for subpopulations of a larger population with a particular disease or condition, or predisposition for a disease or condition; or
 - Advance the competitiveness of the United States in fields of life, biological, and medical sciences; or
 - The Secretary of Labor determines will:
 - Create jobs in the United States.

Relevant Definitions: This section would amend the Internal Revenue Code to add the following definitions:

Personalized Medicine Diagnostic: Cross-reference definition in the Social Security Act (Concept 3).

Personalized Medicine Drug/Biologic: Cross-reference definition in the Social Security Act (Concept 3).

CONCEPT 6: Ensuring Patient Access to Personalized Medicine Through Delivery System Reforms

Center for Medicare and Medicaid Innovation: PPACA established a Center for Medicare and Medicaid Innovation (CMMI) within CMS to “test innovative payment and service delivery models to reduce program expenditures, while preserving and enhancing the quality of care” for Medicare, Medicaid, and SCHIP beneficiaries. This section would require CMMI to consider whether the programs tested by CMMI promote the adoption and use of personalized medicine. In addition, this section would permit CMMI to develop a pilot program to develop and update a clinical decision support system around personalized medicine that would take patient demographic and genetic testing data and compare the results across a database of personalized therapies to determine appropriate interventions, which would be automated to ensure that appropriate updates are made regularly to reflect new advances.

Grants to Provide Personalized Medicine to Medically Underserved Populations. There is a concern that medically underserved populations may have lesser access to innovations in personalized medicine. This section would add a federal grant program to support pilot projects to provide personalized medicine to medically underserved populations, including through the education of patients and providers and the development of culturally targeted interventions for particular populations.

Relevant Definitions: This section would amend the Social Security Act to include the following definition:

Personalized Medicine: Cross-reference definition in Public Health Service Act (Concept 1).