

U.S. Public Policy Inspired by Patients

UCB is committed to the discovery, development, and delivery of differentiated solutions that provide measurable value to patients—improving their lives now and in the future. We're focused on addressing unmet needs and creating sustainable solutions for patients and society. As such, we are dedicated to the continued evolution of a public policy environment that recognizes and rewards **innovation**, encourages **value-based care**, and promotes **affordable access** to medicines for patients. UCB's patient value ambition creates the foundation of our approach to public policy, which aims to:

- Deliver unique **outcomes** that help specific patients achieve their goals;
- Provide patients the best individual **experience**; and
- Ensure **access** to all those who need these solutions in a way which is viable for patients, society, and UCB.

This approach guides our engagement in the healthcare ecosystem and the public policy arena.

Deliver unique outcomes that help specific patients achieve their goals



Innovation

Provide patients the best individual experience



Value-Based Care

Ensure access to all those who need these solutions in a way which is viable for patients, society, and UCB



Affordable Access

INNOVATION

UCB is committed to the development of transformative solutions that treat, modify, or cure severe diseases for patients with unmet medical needs. Therefore, we support policy approaches that:

- Ensure the U.S. Food and Drug Administration (FDA) remains true to its mission¹ of protecting patient safety, and preserve the U.S. system of track and trace;
- Incorporate the patient voice in treatment development and regulatory decision making;
- Support patient-preferred, innovative clinical trial designs;
- Accelerate innovation through incentives for continued investment and the use of real-world evidence (RWE)² as a supplement to randomized clinical trials (RCT) in regulatory decision making;
- Increase the ability of payers to partner with innovators earlier in the drug development process; and
- Advance meaningful protections for innovator intellectual property.

VALUE-BASED CARE

UCB is committed to delivering value-based care for patients through policy approaches that:

- Remove policy barriers to collaboration among patients, payers, providers, caregivers, and policymakers in developing value and outcomes-based payment models;
- Support informed, shared healthcare decision-making between patients and physicians;
- Promote an environment that rewards value and encourages continued medical innovation;
- Incorporate the patient voice and real world evidence (RWE) in healthcare quality measures and value assessments; and
- Foster a value assessment environment that recognizes multiple frameworks and definitions of value.

¹ <https://www.fda.gov/about-fda/what-we-do>

² UCB considers RWE to be any clinical and/or economic findings with respect to disease burden, treatment utilization and outcomes, comparative effectiveness, etc. that analyzes real-world data. Real-world data includes, but is not limited to, medical and pharmacy administrative claims, electronic medical/health records, product/disease registries, and patient-reported outcomes collected through various channels. Methods and analytical approaches (including artificial intelligence) to real-world data collection and RWE generation can vary but acknowledge that RWE must be appropriately executed based on proposed research objectives and be fit for purpose.

AFFORDABLE ACCESS

UCB is committed to promoting affordable access to care for patients through policy approaches that:

- Ensure patients have access to a range of health plan options that meet their individual needs and offer robust protections like transparent, reliable formularies and affordable out-of-pocket costs;
- Remove impediments to providers' ability to prescribe the most appropriate therapy to patients, including step-edits and restrictive formularies, while also reducing patient out-of-pocket expenses; and
- Permit assistance for patients who cannot afford needed medicines.

U.S. Public Policy Inspired by Patients

Innovation

UCB is committed to the development of transformative solutions that treat, modify, or cure severe diseases for patients with unmet medical needs. Therefore, we support policy approaches that:



Ensure that the U.S. Food and Drug Administration (FDA) remains true to its mission¹ of protecting patient safety, and preserve the U.S. system of track and trace;

- We oppose weakening the FDA's preeminent mission to ensure that medical treatments, including prescription drugs, are safe, effective, and secure for patients.

Incorporate the patient voice in treatment development and regulatory decision-making

- We support the inclusion of the patient voice in all aspects of drug development and delivery, including regulatory decision-making, development of patient-reported outcomes, patient-focused drug development forums, and the use of real-world evidence (RWE)³.

Support patient-preferred, innovative clinical trial designs

- We support policies that encourage the creation and adoption of clinical trial designs that minimize the time and cost of drug development by incorporating new technologies to enable decentralized studies that are more convenient and accessible to patients.
- We support increasing the inclusion of patient-reported outcomes in clinical trial designs.
- We support promoting diversity in clinical trials by removing barriers for the inclusion of historically underrepresented subpopulations.

Accelerate innovation through incentives for continued investment and the use of RWE as a supplement to randomized clinical trials (RCT) in regulatory decision making

- We support the further development of policies or pathways such as expedited FDA approval pathways and other incentive frameworks to encourage the development of therapies targeting unmet patient needs in the treatment of a serious or life-threatening condition⁴.
- We support the use of RWE as a supplement to RCT data in regulatory decision-making to expedite clinical development and patient access to new treatments and to ensure that product labels more closely reflect clinical practice.

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Innovation continued...

Increase the ability of payers to partner with innovators earlier in the drug development process

- We support allowing payers to partner with innovators in creating coverage, financing, and access strategies earlier in the drug development and review process, allowing patients to benefit from innovative treatment more quickly upon FDA approval.

Advance meaningful protections for innovator intellectual property

- We support policies that enable a system of intellectual property protections that reward and strengthen core innovation that serves patients, rather than creating IP protection for the sake of creating barriers to competition.

UCB recognizes our obligation to patients, the healthcare ecosystem, and society and is committed to driving sustainable solutions that recognize and reward innovation by:

- Continuing to invest above industry average revenues in the research and development of innovative and transformative medicines;
- Incorporating rigorous, internal value assessments into all phases of our discovery and development to ensure our treatments provide significant and differentiated value;
- Leading the establishment of responsible approaches to sharing clinical trial data that accelerate the development of additional therapies by other healthcare innovators;
- Empowering patients through the development and publication of patient-accessible clinical trial lay-summaries;
- Creating, maintaining, and defending intellectual property of core innovations that deliver real patient and societal value; and
- Promoting a global shift in the approach to intellectual property, innovation, and access through active public policy engagement, patent portfolio transparency, and risk-sharing among healthcare stakeholders.

¹ <https://www.fda.gov/about-fda/what-we-do>

³UCB considers RWE to be any clinical and/or economic findings with respect to disease burden, treatment utilization and outcomes, comparative effectiveness, etc. that analyzes real-world data. Real-world data includes, but is not limited to, medical and pharmacy administrative claims, electronic medical/health records, product/disease registries, and patient-reported outcomes collected through various channels. Methods and analytical approaches (including artificial intelligence) to real-world data collection and RWE generation can vary, but acknowledge that RWE must be appropriately executed based on proposed research objectives and be fit for purpose.

⁴<https://www.fda.gov/regulatory-information/search-fda-guidance-documents/expedited-programs-serious-conditions-drugs-and-biologics>

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Value-Based Care

UCB is committed to delivering value-based care for patients through policy approaches that:



Remove policy barriers to collaboration among patients, payers, providers, caregivers, and policymakers in developing value- and outcomes-based payment models

- We support creating an environment more conducive to value-based care, including by allowing new flexibilities or exemptions from current regulatory barriers such as Medicaid Best Price and the Anti-Kickback Statute.
- We support the continued ability for public and commercial payers to evolve from "one-size-fits-all" formulary designs to value-based insurance design models that improve patient access to the right medicine at the right time.

Support informed, shared healthcare decision-making between patients and physicians

- We support regulatory processes that enable sharing of balanced, straightforward, evidence-based information about medicines that enable patients and providers to make informed healthcare decisions.
- We support patient empowerment through ready access to meaningful and understandable data on their condition and treatment options.

Promote an environment that rewards value and encourages continued medical innovation

- We support evolving the healthcare ecosystem by removing barriers to private market solutions that foster continued innovation, protect patient access, and promote the development of new life-changing medicines.
- We oppose inhibiting competition, innovation, and sustainable value creation, including arbitrary government price-setting, pricing based on foreign markets, or proposals that require the disclosure of proprietary information.

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Value-Based Care continued...

Incorporate the patient voice and real-world evidence (RWE)⁵ in healthcare quality measures and value assessments

- We support integrating the patient voice, patient-reported outcomes, and RWE into value assessment frameworks and quality measurement.
- We support increased recognition of the differentiated value of medicines that modify or cure underlying disease.
- We strongly oppose quality measures or value assessment frameworks that diminish or discount the value of human life based on age, gender, disability or other factors.

Foster a value assessment environment that recognizes multiple frameworks and definitions of value

- We support the development and use of multiple value assessment frameworks and the recognition of a variety of definitions of what constitutes value to patients, providers, payers, caregivers, and society.

UCB recognizes our obligation to our patients, the healthcare ecosystem, and society, and is committed to driving sustainable solutions that deliver value-based care for patients by:

- Collaborating with patient organizations to identify and incorporate the outcomes of greatest significance to patients into value assessment frameworks, with specific effort to include subpopulations with unique unmet needs.
- Partnering with public and commercial payers on value- and outcomes-based pricing and contracting models for our medicines.
- Investing in the continued development of evidence that demonstrates the value of our medicines to the Food and Drug Administration (FDA), the Centers for Medicare and Medicaid (CMS), private payers, providers, and, most importantly, patients.
- Working to improve the sustainability of our company, the overall healthcare ecosystem, and the environment.

⁵UCB considers RWE to be any clinical and/or economic findings with respect to disease burden, treatment utilization and outcomes, comparative effectiveness, etc. that analyzes real-world data. Real-world data includes, but is not limited to, medical and pharmacy administrative claims, electronic medical/health records, product/disease registries, and patient-reported outcomes collected through various channels. Methods and analytical approaches (including artificial intelligence) to real-world data collection and RWE generation can vary, but acknowledge that RWE must be appropriately executed based on proposed research objectives and be fit for purpose.

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Affordable Access

UCB is committed to promoting affordable access to care for patients through policy approaches that:



Ensure patients have access to a range of health plan options that meet their individual needs and offer robust protections like transparent, reliable formularies and affordable out-of-pocket costs

- We support ensuring availability of affordable health insurance options that provide robust benefits, access to clinically appropriate medicines or treatments, and rigorous patient protections.
- We support requirements to provide patients with easy-to-understand information about their pharmacy and medical coverage, out-of-pocket costs, and potential care restrictions to support informed decision-making.
- We support guaranteeing the terms of health insurance coverage, including the prohibition of mid-year formulary changes that create new restrictions to treatment access.
- We support requirements around utilization management techniques to ensure clinical appropriateness and transparency to both the patient and physician.
- We support patient affordability measures including annual limitations on out-of-pocket costs and policies that pass rebates and discounts from drug manufacturers to patients at the pharmacy counter.

Remove impediments to providers' ability to prescribe the most appropriate therapy to patients, including step-edits and restrictive formularies, while also reducing patient out-of-pocket expenses

- We support providers' ability to determine appropriate treatments for their patients without undue influence (control) from health plans; therefore, we promote access to simple, easy-to-use frameworks for overriding formulary restrictions such as step therapy and prior authorization.

Permit assistance for patients who cannot afford needed medicines

- We oppose policies that penalize patients for accepting drug manufacturer assistance in affording their medicines.

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Affordable Access continued...

UCB recognizes our obligation to our patients, the healthcare ecosystem, and society, and is committed to driving sustainable solutions that deliver value-based care for patients by:

- Applying a consistent approach to pricing our medicines based on maximizing access for patients while balancing patient affordability, UCB innovation, and sustainability;
- Supporting transparency by sharing pricing and affordability information—including estimated patient out-of-pocket costs—on our website;
- Providing support and solutions for patients who cannot afford their medicines;
- Investing in programs to enhance the patient experience, including financial assistance and educational support designed to improve patient adherence and stability; and
- Offering programs to support expanded access to investigational medicines under specific circumstances for patients with severe or life-threatening conditions.