BERLIN INSTITUTE OF HEALTH

Personalized Medicine, Advanced Therapies

Maintaining Health and Quality of Life, Creating Value: BIH Strategy 2026



"By 2026, we will have established a robust ecosystem where innovation is a driving force for better health and quality of life."



Prof. Erwin BöttingerChief Executive Officer



Dr. Rolf ZettlChief Financial Officer



Prof. Karl Max Einhäupl Charité – Universitätsmedizin Berlin, Chief Executive Officer



Prof. Axel Radlach Pries Charité – Universitätsmedizin Berlin, Dean



Prof. Martin Lohse
Max Delbrück Center for
Molecular Medicine in the
Helmholtz Association,
Chair of MDC Board

WELCOME

The developed and developing worlds alike are increasingly burdened by noncommunicable diseases. Illnesses such as heart disease, cancer and dementia have a huge impact on our societies causing untenable morbidities and mortality. With people now living longer and expecting better health and quality of life in old age, a new approach is needed.

We believe the emerging fields of personalized medicine and new advanced therapies offer a promising way forward. Since the year 2000, great strides have been made in biomedical research, not least the unraveling of the human genome, advances in stem cells and cell-based therapies, and new prospects for gene therapy through genome editing technologies. Coupled with advances in digital medicine and information sciences, these innovative fields have created huge opportunities to develop new ways of tackling in particular progressive forms of noncommunicable disease.

The Berlin Institute of Health aims to create an innovative translational research commons of Charité – Universitätsmedizin Berlin and the Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC). The BIH Research Commons is enhancing personalized medicine and advanced therapies research in Germany

and aims to develop predictive tools for personalized medicine as well as advanced therapies that save lives and prolong quality of life. It is not the first of its kind, but we want to make it among the best.

The new Board is now leading BIH into an exciting phase. Our job is to build on solid foundational work to grow the institute and meet the objectives laid out in our ten-year BIH Strategy 2026, which this booklet communicates. We are ambitious. In just a few years we want to have recruited the brightest and most ambitious faculty and staff, created multiple research initiatives covering emerging areas from digital medicine to cell engineering and perhaps even have already developed new diagnostics or therapies. And by 2026, we will have established a robust ecosystem where innovation is a driving force for better health and quality of life.

Our approach will always be integrated, forward thinking and research driven: our aim always to make the best possible use of life and data sciences for personalized medicine and advanced therapies.

Welcome to future medicine.

The Executive Board of the Berlin Institute of Health

Personalized medicine facilitates tailored health care instead of "one-size-fits-all". The concept is not new: Clinicians have long known that medical treatments may work well in some patients with a disease but not in others with the same disease. What is new is that advances in a wide range of fields from genomics to stem cells to regenerative medicine, along with data science are allowing patients to be treated more effectively and in ways that better meet their individual needs.

14th

The MDC's ranking in the Thompson Reuters list of the world's 20 best research institutes for molecular biology and genetics.

Personalized medicine creates added value for all in the healthcare industry:

- **▶** Patients
- **▶** Clinicians
- **Entrepreneurs**
- ▶ Regulatory authorities
- **▶** Payers

Growth in the number of personalized medicine market volume worldwide

≈11.8%

annually

from just over \$1 billion in 2014 to over 2.4 billion in 2022. (Grand View Research, 2016) Interdisciplinary approaches and new scientific alliances are crucial for success in translational medicine.

=150 million

third-party funding each year secures Charité – Universitätsmedizin Berlin's leading position among university hospitals in Germany

BIH's **5 ways** to maximize the efficiency with which research results are translated into applications:

- 1. Excellence and Quality in Science
- 2. Education and Training
- 3. Services, Open Science, Infrastructure
- 4. Public Outreach and Involvement in Research
- 5. Spirit of Entrepreneurial Research

BIH builds on the joint forces of more than

4,000

researchers and clinicians at MDC und Charité

The Berlin Institute of Health has two key research priorities: to improve personalized outcome prediction in progressive diseases and to develop advanced therapies for personalized treatment.

INTRODUCTION

Personalized medicine, Advanced therapies

We hear about breakthroughs in medical research almost every day. But often the period before such findings are tested and made available to patients is excessively long. So imagine a world where promising, safe new treatments are swiftly brought out of the lab, quickly disseminated and speedily used to improve quality of life. Better still, imagine if they could be tailored to specific patient groups, or even used to heal individuals with their own cells.

These are the exciting new worlds of personalized medicine and advanced therapies, and the Berlin Institute of Health aims to lead both to turn the tide against the most burdensome progressive diseases.

THE BERLIN INSTITUTE OF HEALTH

BIH is a cutting-edge translational research institute. It was launched as a joint initiative in 2013 by the Charité – Universitätsmedizin Berlin and the Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC). The Charité is the top-ranked university hospital in Germany and one of the largest in Europe, excelling in the fields of medicine and research. The MDC, with more than 80 senior researchers, ranks among the world's 20 best research institutes for molecular biology and genetics.

Our goal is simple: to exploit collaboration between both organizations and together with international partners to turn innovative advances in biomedical research into tangible health benefits.

Having completed our foundational phase, BIH is now moving quickly to establish itself as a leading translational medicine research center. By 2026, we will integrate hundreds of staff across four Berlin campuses with an annual estimated budget of more than €100 million.

WHAT WE DO

Translational medicine links research with patient care to help quickly deliver new treatments and predictive tools. Personalized medicine focuses on tailoring treatment plans to patient groups or individual patients with different needs. Advanced therapies are typically custom-tailored to the individual patient and include emerging fields of regenerative medicine, cell- and gene-based therapies and nanoscale therapies. BIH is combining these innovative fields to study, for example, how progressive diseases such as some heart disease, cancer and dementia develop, and find new treatments for them.

OUR MISSION

BIH's mission is to maintain or restore the health and quality of life for people with progressive diseases. We will achieve this by providing research and clinical solutions that enable the transformation of medical care into a personalized, value-based model. This booklet, based on our strategy, outlines our plans.

CHALLENGES AND PRIORITIES



The increasing burden of progressive diseases is requiring a fundamental shift in their treatment, focused on areas such as digitization, patient involvement and advanced therapies.

CHALLENGES

The new healthcare landscape

Health and healthcare have a fundamental role in our well-being, economy and standard of living. But across the industrialized world, healthcare systems are facing a fundamental shift.

People are living longer, and as a result the burden of chronic-progressive and chronic disease is increasing. Healthcare costs are rising and quality of life and productivity is jeopardized. This is necessitating a farreaching change in the way patients are treated.

Studies have shown that linking healthcare spending to improved quality and quantifiable outcomes – so-called value-based programs – achieves better results at lower cost. Also, as people become more informed and concerned about staying healthy, and researchers learn more about risk factors, the concept of tailoring healthcare to particular needs is gaining ground. The problem is, research is struggling to keep up. Deficiencies are emerging in the quality and value of biomedical research. This is resulting in a shortage of new therapeutic approaches.

SOLUTIONS

Such huge challenges are not easily overcome. But recent studies have identified key areas that could tip the balance. These include:

Digitization

To be effective, translational and personalized medicine must be able to draw on digital clinical information systems and patient health records.

Patient involvement

Shared responsibility for healthcare and health research must be encouraged to ensure patients are involved in disease prediction and therapies, and become engaged partners in research.

Systems medicine research

Health research must be concentrated into research on how bodily systems interact (systems medicine) to advance personalized medicine.

Advanced therapies

Technologies such as gene, cell and nanoscale therapies must be developed to tackle progressive disease in individuals.

Innovation

Value-based healthcare systems require a cultural and behavioral shift among researchers, including improved technology transfer practices and entrepreneurial training to bring new developments faster to patients. BIH strives to enable and elevate a research commons of excellence where researchers of Charité and MDC join forces with newly-recruited research groups along the programmatic framework.

(2)

PRIORITIES

Solutions to save and improve lives

BIH has spent its foundational phase analyzing the new healthcare challenges and developing solutions for them. As such, our new Strategy 2026 is built around them. It aims to meet our mission of maintaining or restoring the quality of life of people with progressive diseases by developing diagnostic tools and advanced therapies where they are most needed or do not already exist (so-called unmet medical needs).

STRATEGIC PRIORITIES

Most people probably know someone who suffers from a progressive disease. They include heart disease, dementia, certain types of arthritis, Parkinson's disease, kidney failure, cancer, and many others. All severely affect the lives of the affected, and often have multiple complications.

Yet our ability to treat them is often hampered by a lack of understanding in the variability of the diseases, patient responses to standard treatments and of the possibilities of new therapies. This means that patients often have unmet medical needs.

With this in mind, BIH's strategy will prioritize two key areas:

(1) Improvement of outcome prediction in progressive diseases for personalized medicine based on systems medicine research

Development and use of advanced therapies for the personalized treatment of progressive diseases in cases of unmet medical needs

COLLABORATION, INTEGRATION, INNOVATION

The two strategic priorities will be the glue that binds BIH's research and operations together. Two research programs will be implemented to mirror them, with each supported by four shared research platforms. To further maximize impact, twin innovation drivers focused on technology transfer, transformation and training will run through all of these structures to remove translational barriers and foster a spirit of entrepreneurial research.

The graphic of the BIH Translational Research Commons shows how these strands will mesh together, and the programs, platforms and innovation drivers are described in detail over the following pages.

BIH's programs, platforms and innovation drivers make up the translational research commons of Charité – Universitätsmedizin Berlin and the Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC)



STRATEGY



SUMMARY

Based on its priorities, BIH will focus on two core research programs: Studying how progressive diseases develop and producing advanced therapies. Core research programs will draw dynamically on scientific-technological development of four research platforms.

STRATEGY

Research programs: The focus

To reflect our two priorities, BIH's research is focused on two core programs: personalized medicine for progressive diseases; and advanced therapies for progressive diseases.

PERSONALIZED MEDICINE FOR PROGRESSIVE DISEASES

Understanding how progressive diseases develop, both in general and specific cases, is key to finding treatments for them.

OBJECTIVE

To develop and apply better markers and models for predicting and understanding progressive disease.

APPROACH

Interdisciplinary research groups will use cutting-edge systems-medicine techniques to investigate the role and interactions of failing biological systems in progressive diseases. This approach is unique in that it will focus on comprehensive – "deep" – characterization of patients, comprehensive studies of the make up and interplay of biological systems and big data analyses.

DEVELOPMENT

Research initiatives within the program will be promoted through targeted funding, infrastructure and recruitment of researchers.

ADVANCED THERAPIES FOR PROGRESSIVE DISEASES

Advanced therapies, such as nanoscale, gene and cell therapies, offer great potential for improving the lives of people with progressive diseases by helping to replace or regenerate damaged tissue.

OBJECTIVE

To promote, through interdisciplinary research, the development of advanced therapy technologies and their personalized application in patients with progressive diseases.

APPROACH

Research initiatives will focus on developing new therapies for use in replacement or regeneration of damaged tissues, genetic modification of stem cells for treatment of rare genetic disorders and cell-based personalized tumor-targeting immunotherapy.

DEVELOPMENT

Research into advanced therapies, in particular regenerative therapies, is already underway at BIH, Charité and the Max Delbrück Center for Molecular Medicine. BIH will further merge and develop these areas, targeting funding at new state-of-the-art facilities and the recruitment of researchers.

BIH's four research platforms will dynamically support the two research programs and focus on digitization, patient involvement in research, multiscale genomics, and human models/cell engineering.

Research platforms: The building blocks

BIH's four research platforms will be anchored by center-like units where innovative technologies, methods and research structures are developed to support the two core programs. They focus on digital medicine; clinical translational sciences; multiscale genomics; and humanized model systems and cell engineering. Each will build on or draw together existing facilities in the transational research commons and interact with additional structures of Charité and MDC.



DIGITAL MEDICINE

WHY?

Digitization is transforming research and healthcare. The introduction of electronic health records and digital clinical information systems has helped to revolutionize the quality of healthcare in countries such as the United States. Linking clinical data with information from insurers on people's fitness and even about their genes allows clinicians to make informed decisions in real time, and enhances the development of personalized medicine. Germany, especially, has much to gain in this area.

HOW?

BIH's Digital Medicine Platform therefore plans to combine its ongoing work in this area and that of partner institutes to improve data use in research and clinical practice.

We will do this by establishing a Digital Health Center and provide a hub for the Digital Medicine Platform, focused on key areas such as health informatics, artificial intelligence and big data. Its activities will include, for example, developing IT infrastructure, big data analytical tools and mobile healthcare apps.

The platform will support all the initiatives within BIH's translational research commons and be directed by two BIH chairs and five professorships by 2026. Its aim is to help connect sources of health data in Germany and ultimately establish a leading digital medicine center in Berlin.

58%

of US smartphone users have downloaded a health-related app, according to a 2015 study by NYU Langone Medical Center. (Roland Berger analysis)

CLINICAL TRANSLATIONAL SCIENCES

WHY?

In the future, patients will play a significantly more active role in helping researchers formulate important research questions, and will often benefit directly from the results of their personal study data. This will lead to significant advances in clinical research approaches and methods.

HOW?

Work on such clinical and translational scientific activities is already well underway in several BIH and Charité-led bodies, such as BIH's Clinical Research Unit and Charité's clinical research centers. The Clinical Translational Science Platform, anchored by the Clinical Science Center, will enhance the translational research commons in an integrated environment with these facilities.

It will consist of a number of scientific and management units designed to support the next generation of clinical translational research. These will include a consolidated clinical research unit; an advanced biobank to manage the storage of biosamples; infrastructure to produce advanced therapies; and a specialist unit that will develop and share new clinical study approaches and methods.

The platform will be supported by two BIH chairs and five professorships with expertise in comparative effectiveness, health economics, epidemiology, clinical trials and methods development. Its aim, in addition to supporting the translational research commons, is to conduct world-class research and become scalable to link with similar centers across Germany and internationally.

More than

70%

of the general public thinks that patients need more opportunities to participate in clinical trials. (PACE Cancer Perception Index)





>25,000
The number of genotype-phenotype

associations revealed through genomics.

MULTISCALE GENOMICS

WHY?

Ever since the human genome was decoded in 2003, genomic technology has exploded. A person's DNA can now be sequenced for less than €1,000, meaning their genetic material can readily be analyzed for potential disease susceptibilities or gene therapy targets. The development also presents huge research opportunities, not least using models with multiple layers of molecular biological and clinical data to understand how genes and mechanisms that control their function affect disease (multiscale genomics).

HOW?

Several translational research units within BIH and its corporate bodies are active in this area, creating an ideal situation for a coordinated "Berlin approach" for multiscale genomics focused on translation. BIH's plan is therefore to establish a Multiscale Genomics Platform anchored by a Translational Genomics Center in complementary interaction with Charité and MDC structures. This will encompass a medical genomics unit to investigate the genetic causes of disease; a systems medicine/multiscale genomics/bioinformatics unit to study disease causation; and a unit looking at the role of microorganisms in health and disease.

These will build on existing BIH facilities and be enhanced through the recruitment of one chair and five professorships, specializing in areas including population genetics, genetic epidemiology and systems biology. The platform aims to become an international beacon for translational genomics and personalized medicine.

300

The approximate number of human embryonic stem cell lines available worldwide.

HUMANIZED MODEL SYSTEMS AND CELL ENGINEERING

WHY?

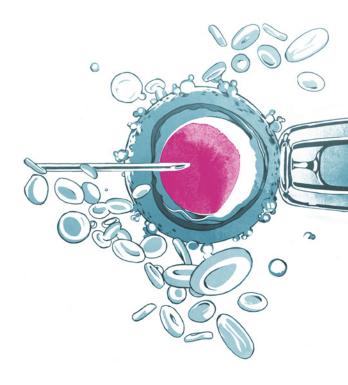
Stem cell technologies and gene editing techniques are two of the most promising recent developments in biomedical science. The ability to reprogram common cells, such as those in blood, and turn them into the multipurpose cells the body uses to regenerate tissue has already become a powerful research tool and has great therapeutic promise. And the use of enzymes to alter gene sequences, for example by repairing disease-causing mutations, could soon be widespread. Both are valuable in developing personalized disease models and personalized therapies alike. They are complimented by new methods of growing and testing human tissue in mice – humanized models.

HOW?

Charité and MDC conduct internationally recognized research in these areas and they plan to further develop it in the BIH Translational Research Commons through the establishment of a Stem Cell and Disease Model Center to anchor its Humanized Model Systems and Cell Engineering Platform.

In particular, the platform will support researchers who carry out patient-specific stem cell studies; test new human models for stem cell research; further develop gene-editing techniques; and develop and design humanized models in such areas as cross-species transplantation and "organs-on-a-chip".

The work will be carried out under the guidance of two BIH chairs and six professorships. The platform aims to conduct research at the highest level.



BIH will establish a Center for Transforming Biomedical Research, a Biomedical Innovation Academy and a Technology Transfer Unit ("Berlin Health Innovations") to promote innovation and value creation.

Innovation drivers: The value generators

The physical process of bringing new ideas and promising therapies to fruition is one of the toughest challenges in biomedical research, and one of the most important for a translational medicine center to get right. Researchers must know what to do with promising results, have a clear pathway to follow, and have expertise to support them, for example, in bringing a therapy to market.

In the past, this process of driving innovation through so-called technology transfer and specialist entrepreneurial training has been underplayed in Germany. BIH therefore plans to take a new holistic approach that focuses on value creation, fostering a culture of innovation, commercializing assets and developing a network of expertise. This will consist of two interlinked functional units, led by one director and one chair, that permeate research programs and platforms of the BIH Translational Research Commons.

"We really need to be ambitious here."

Dr. Rolf Zettl, chief financial officer and responsible for technology transfer

TECHNOLOGY TRANSFER

The purpose of BIH's technology transfer unit will be to remove obstacles that exist in the research-to-clinical-practice pipeline. By joining the Charité's technology transfer team we will establish "Berlin Health Innovations", one-stop-shop to commercialize assets, closely aligned to the needs of healthcare industries. It will provide advice, funds and commercial know-how to researchers, eventually offering a network of internal and external advisers.

RESEARCH DEVELOPMENT AND TRAINING

To ensure researchers spot opportunity and optimize the potential of their work, they must be properly trained in effective methodologies. To facilitate this, BIH will set up a Center for Transforming Biomedical Research (CTBR) and a Biomedical Innovation Academy (BIA) within a close-knit second functional unit.

The CTBR will seek to devise and evaluate methods of optimizing scientific practices, and of advancing open science, citizen science, and bioethical frameworks to enhance value for all stakeholders through transforming biomedical research.

The BIA's aim is to develop a new generation of translationally and entrepreneurially trained researchers. At its heart will be a new cross-discipline biomedical entrepreneur career path for clinician scientists and basic researchers.

SUMMARY

BIH will recruit several chairs and numerous professorships and junior group leaders by 2026, always seeking to attract the brightest minds in translational research.

Recruitment strategy: The people factor

OUR RESEARCHERS AND STAFF

People are at the heart of BIH's work, and we aim to attract, develop and retain the brightest minds of translational research and management. We already have dedicated researchers and staff, but to meet our Strategy 2026 goals, we plan to recruit more by applying proven best-practice approaches and embracing gender equality and internationalization. We aim to have filled dozens of key posts for the translational research commons by 2026, from BIH chairs to BIH professorships and junior group leaders. The chairs will be research leaders with exceptional academic track records. They will build our research programs and platforms, spearhead innovative new research centers and aid recruitment. BIH professorships will conduct independent research at the highest level and distinguish themselves through internationally recognized achievements. They will also enhance and strengthen our impact. Junior group leaders will be promising scientists in an early stage of their careers who can be expected to earn an international reputation. Our business administration teams will be key to meeting our objectives. They will manage vital areas such as technology transfer, communications and recruitment, and ensure the smooth running of the BIH Research Commons.

OUR RESEARCH COMMUNITY

BIH-supported researchers will work with colleagues at Charité and MDC, so-called BIH members, to excel in the translational research commons. All will participate in BIH activities, working to the same standards and objectives, and be able to access BIH funds. Through this extended community, BIH aims to establish an inclusive and open translational research commons that permeates its corporate bodies Charité and MDC with maximal impact for all members.

PROFILE: WHAT OUR STAFF DO



Professor Holger Gerhardt Head of Integrative Vascular Biology at MDC and BIH Scientific Committee member

Holger Gerhardt moved from the London Research Institute, a major cancer center, to take up a BIH professorship in September 2014. He and his team are looking at the role blood vessels play in disease, with a particular focus on cardiovascular conditions. "Our main aim is to unravel the fundamental mechanisms of how blood vessels form," he said. "The challenge then is to understand how this is important for the complications that patients have." Blood vessels play a key role in many progressive diseases. "Having ready access to BIH resources such as patient materials, stem cell facilities and gene sequencing will be key to our work." He describes the close bench-to-bedside link at BIH, MDC and Charité as "super exciting".



Birte KehrJunior Research Group Leader in Bioinformatics

Birte Kehr joined BIH in November 2016. With her team she develops algorithms for identifying genomic variation from sequence data. "Our main goal is to improve the detection and genotyping of structural variation," she said. This is important since variation in a person's genomic sequences influences susceptibility to disease and response to medical treatment. "I'd like to see our algorithms being used routinely in a clinical setting one day," she said.

FACTS & FIGURES

LEGAL FRAMEWORK

The Berlin Institute of Health was initially founded as an internal GbR (a partnership under civil law) in 2013. Since April 2015, BIH is a "Körperschaft des öffentlichen Rechts" (public corporation) and thus independent and vested with legal capacity. Charité – Universitätsmedizin Berlin and Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC) are independent, member entities within BIH. The law for the establishment of BIH was passed by the Abgeordnetenhaus Berlin (Berlin House of Representatives) in March 2015.

FUNDING

BIH receives institutional funding from the German federal government and the state of Berlin in a ratio of 90:10. The funding for the upcoming period 2017–2020 will be approximately 290 million euros. BIH is also supported by the Private Excellence Initiative of Johanna Quandt with additional 40 million euros by 2022. The funds are managed by the Charité Foundation.

INSTITUTIONAL MILESTONES

Memorandum of Understanding
Scientific Concept
Administrative Agreement (Verwaltungsvereinbarung)
Founding Agreement (Gründungsvertrag)
BIH is a civil law association (Innengesellschaft bürgerlichen Rechts)
Evaluation of Scientific Concept
Berlin State Assembly passes BIH Legislation
BIH operates as independent corporation under public law
Supervisory Board approves "BIH Strategy 2026"
Supervisory Board approves "Strategy Implementation Plan 2017–2020"
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LEADERSHIP | EXECUTIVE BOARD

Prof. Erwin Böttinger (Chief Executive Officer)

Dr. Rolf Zettl (Chief Financial Officer)

Prof. Karl Max Einhäupl (Charité –

Universitätsmedizin Berlin, Chief Executive Officer)

Prof. Axel Radlach Pries (Charité –

Universitätsmedizin Berlin, Dean)

Prof. Martin Lohse (Max Delbrück Center for Molecular Medicine in the Helmholtz Association, Chair of MDC Board and Scientific Director)

SCIENTIFIC ADVISORY BOARD

The Advisory Board is responsible for supporting the Board and the Supervisory Board in scientific and strategic matters with external expertise. It consists of 14 internationally acclaimed scientist, including several Nobel Laureates.

SUPERVISORY BOARD

Activities of BIH are supervised by the Supervisory Board, which is also responsible for approving essential decisions such as the business plan, strategic planning, appointments and investments.

MEMBERS & COMMUNITY

The members of the BIH are professors employed full time by the Charité (professors, junior professors), as well as research group leaders of the MDC. As of November 30, 2016, more than 250 research staff were involved in various research initiatives. To successfully meet objectives of its Strategy 2026, BIH is extensively recruiting chairs, professorships and junior group leaders.

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Photos:

(Page 3, from left to right)
BIH, Thomas Rafalzyk
Helmholtz Association, David Ausserhofer
BIH, Tom Maelsa
Charité – Universitätsmedizin Berlin
Nikolaus Brade
(Page 17)
MDC, David Ausserhofer
Birte Kehr

ABOUT BERLIN INSTITUTE OF HEALTH

The Berlin Institute of Health (BIH) is a biomedical research institution focusing on translational research and precision medicine. BIH is dedicated to improving the prediction in progressive diseases and developing advanced therapies in order to improve patients' health and quality of life. The institute is committed to providing excellent research solutions and innovation enabling value-based, personalized healthcare. The two founding institutions, Charité – Universitätsmedizin Berlin and Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC), are independent, member entities within BIH.

Berlin Institute of Health Kapelle-Ufer 2 10117 Berlin Germany www.bihealth.org/en info@bihealth.de

@berlinnovation



