BIOMEDICAL SCIENCES
Development of Cell, Drug and Gene Therapies

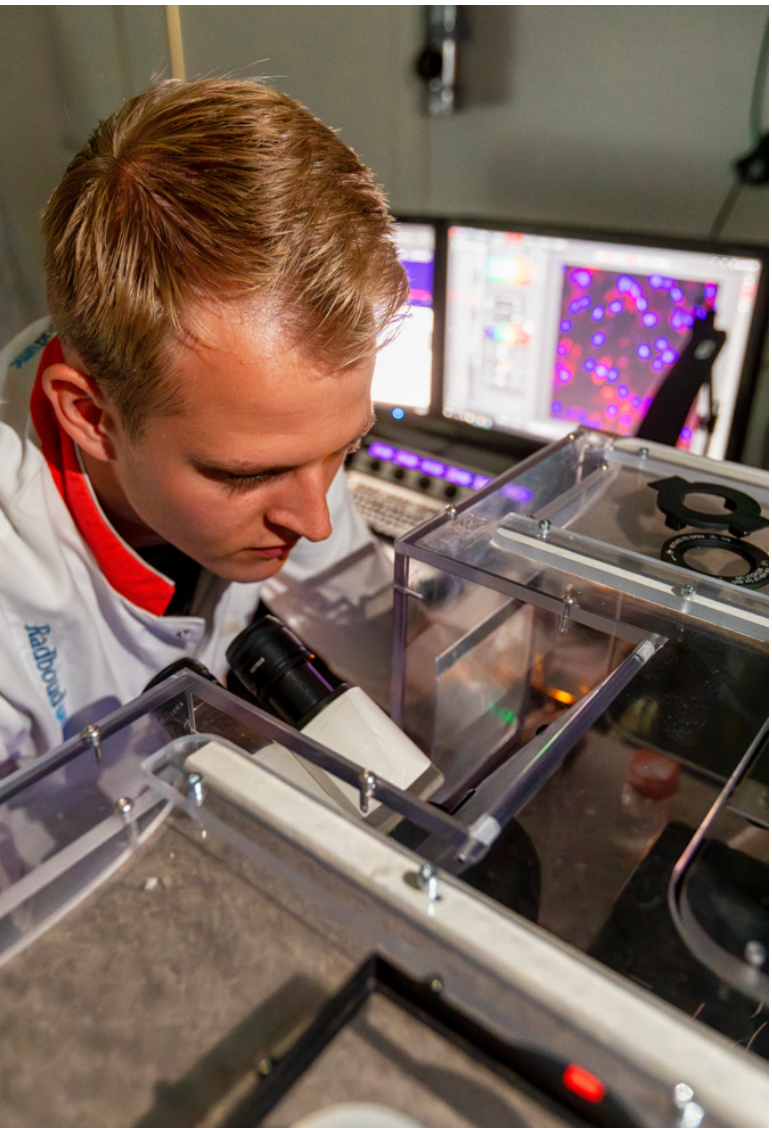
Radboud Universiteit Radboudumc

Development of Cell, Drug and Gene Therapies

A specialisation in the Biomedical Sciences Master's

From innovation to impact:

Explore the multidisciplinary landscape of developing novel therapies (including cell, gene and repurposed modalities) and discover how to navigate pathways to bring these treatments to patients, with special attention for the development of therapies for rare diseases.



Specialisation Coordinators

Dr. Rick Greupink
Department: Pharmacy, Pharmacology & Toxicology
Email address: Rick.Greupink@radboudumc.nl

Dr. Najoua El Boujnouni
Department: Medical BioSciences
Email address: Najoua.ElBoujnouni@radboudumc.nl

The BMS Master's

Our Master's programme in Biomedical Sciences offers eight specialisations and four career profiles. In the first semester, each specialisation starts with four courses that reflect its central topics and methodology, followed by an elective course. In the second semester of the first year students do a research internship.
In the second year students follow elective courses and courses of their career profile, followed by a profile internships.
Furthermore, the course 'Personal & Professional Development' runs throughout the entire programme under the guidance of a coach. It covers topics such as personal learning goals, responsible research & innovation, well-being, and career prospects.

The specialisation

Development of Cell, Drug, and Gene Therapies (CDGT) is designed to equip Biomedical Sciences master's students with the expertise needed to navigate the complex journey of CDGT development. To learn how to bring drugs and innovative therapies to (even few) patients, the entire pipeline will be covered, from preclinical research and clinical trials to regulatory approval, as well as ethical and economic considerations. With this foundation, you will be well equipped to contribute across multiple sectors, including academic, industrial, and regulatory settings. Examples of questions to be addressed:

- Which emerging molecular and cellular therapeutic tools are available, and how can these be implemented as therapies?
- Which preclinical and clinical methods and techniques can be used to test the potential of these innovative therapies?
- What are the unique hurdles in developing therapies for rare diseases?
- How does one set-up a clinical trial for small patient populations or use innovative in silico, in vitro and in vivo preclinical strategies to learn about the new drug?
- How do regulatory requirements and pathways shape the development process and approval of advanced therapies?
- How do ethical, economical and societal considerations influence development of therapies and patient access?

Internship possibilities

You will choose a research, consultancy or communication profile and you will do two internships of your own choice. One research internship and one profile internship. Internships in the field of Development of CDGT can be done at different world renowned research groups from various departments of the Radboudumc, but there are also plenty of opportunities at other locations in the Netherlands or abroad. We have many connections with research institutes, companies, and universities all over the world. So the possibilities are endless!

Career perspective for Development of Cell, Drug and Gene Therapies alumni

Graduates can pursue careers as PhD candidates and researchers in academic hospitals, universities and research institutes, that often focus on innovative therapies for complex diseases. Outside academia, they may work in the pharmaceutical and biotech industries as scientists, project managers, or regulatory affairs specialists, contributing to the development and approval processes of CDGT. With their multidisciplinary expertise, alumni are also well-suited for roles in clinical trial coordination, healthcare consultancy, and policy-making, helping to advance biomedical innovations and improve patient care.

Specialisation courses

DRUG DEVELOPMENT & SAFETY

This course offers an essential introduction to the foundational principles of drug development, pharmacology and pharmaceutical toxicology, offering an overview of the development pipeline of cell, drug and gene therapies, while emphasizing the critical importance of both efficacy and safety in the development of therapeutics. Reflecting the core medical principle of “first, do no harm”, the course explores how this principle guides every stage of developing new therapeutic modalities. Students will gain insight into the scientific and regulatory considerations that underpin the translation of laboratory discoveries into effective and safe patient treatments.

PRECLINICAL DEVELOPMENT OF CDGT

This course provides a solid foundation in early-stage drug development of CDGT, including essential steps required to advance innovative therapies from the laboratory to clinical trials. The regulatory requirements for preclinical studies, including the demonstration of proof-of-principle and safety are emphasized and students will examine advanced disease models, methods for efficacy evaluation, translational biomarkers, and the challenges associated with in vivo drug and gene delivery. The course focusses on emerging therapeutic modalities such as cell and gene therapies, RNA-based treatments, and drug repurposing, particularly regarding their potential in treating rare diseases. By the end of the course, you will be able to critically evaluate and design preclinical studies with strong translational relevance.

Internship example: antisense therapy

A myotonic dystrophy type 1 patient participating in a phase 1/2 clinical trial, showing his muscle biopsy scar while receiving an infusion of RNA modulatory therapy. The treatment consists of an antisense oligonucleotide conjugated to an antibody fragment that targets transferrin receptor 1, which is expressed in muscle tissue.

CLINICAL DEVELOPMENT OF CDGT

In this course, you will explore the regulatory frameworks and clinical methodologies to translate preclinical discoveries into safe and effective therapies for patients. Students will learn which types of clinical studies are required to ensure patient access to new therapies and how these studies should be optimally set-up. The course covers the fundamentals of clinical trial design and execution, with particular attention to innovative approaches such as basket trials and n=1 trials, as well as dose selection strategies grounded in pharmacokinetics and pharmacodynamics (PK/PD) to address the needs of specific patient populations. Additionally, the course delves into formulation and Good Manufacturing Practice (GMP) requirements essential for first-in-human studies. Emphasizing the unique challenges and opportunities in rare disease research, real-world examples are used to illustrate both the potential and complexities of therapeutic intervention strategies. By the end of the course, you will be able to critically assess and design clinical development programs that advance innovative therapies from the laboratory to the patient.

ECONOMICS & ETHICS OF CDGT APPROVAL

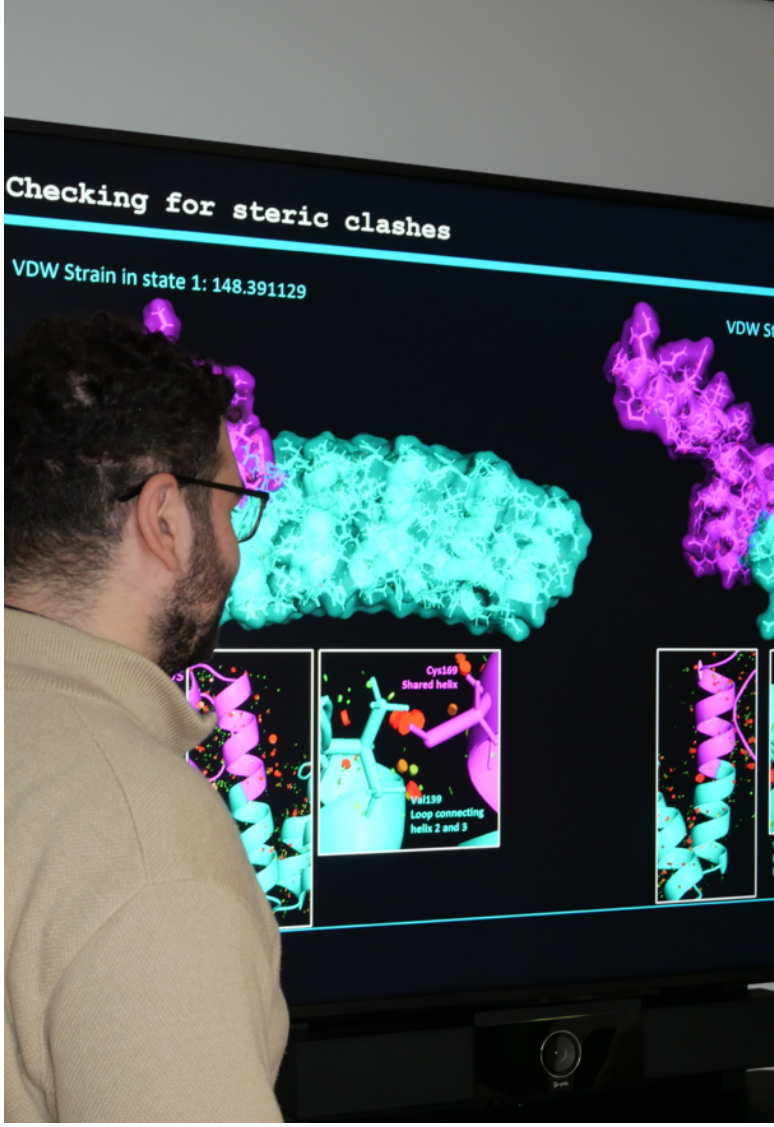
In this course, the critical roles of health technology assessment (HTA), drug pricing, intellectual property management, and valorization strategies in the translation of scientific advances into accessible treatments will be explored. Through engaging case studies, debates, and direct interaction with experts, patients, and advocacy organizations, you will develop the ability to evaluate the ethical, societal, and economic implications in the development of CDGT. By building on foundational knowledge from earlier courses and fostering interdisciplinary dialogue, this course examines the development landscape from multiple perspectives, allowing you to gain a thorough understanding of the key factors that drive the successful development of novel therapies.

ELECTIVES

Interested in delving deeper into a specific topic or exploring other fields? The master's program offers a range of electives related to Biomedical Sciences.

Other courses

In your elective space you can take additional courses at other faculties or universities if you want. Many students choose to do courses outside of the Radboudumc, for example at the other programmes with a focus on innovative therapies in the Netherlands. Also, some students choose to go abroad to follow courses.



Internships: Broaden your perspective on the development of cell, drug and gene based therapies

Interested in an internship outside of academia? As a specialisation, we encourage you to explore the different avenues in the development of CDGT. Therefore, we stimulate internships at regional biotech companies, where you can contribute to the development of drugs in an industry environment, or with regulatory or governmental authorities involved in the legislation and surveillance of treatments in our healthcare system.

Please email us for more information about the programme, the specialisation or the application process.
Admissions@radboudumc.nl

For general information, or a chat with current students, please visit our website.
www.ru.nl/masters/bms

RU-drxa23fbbeq-en-US-0424-2