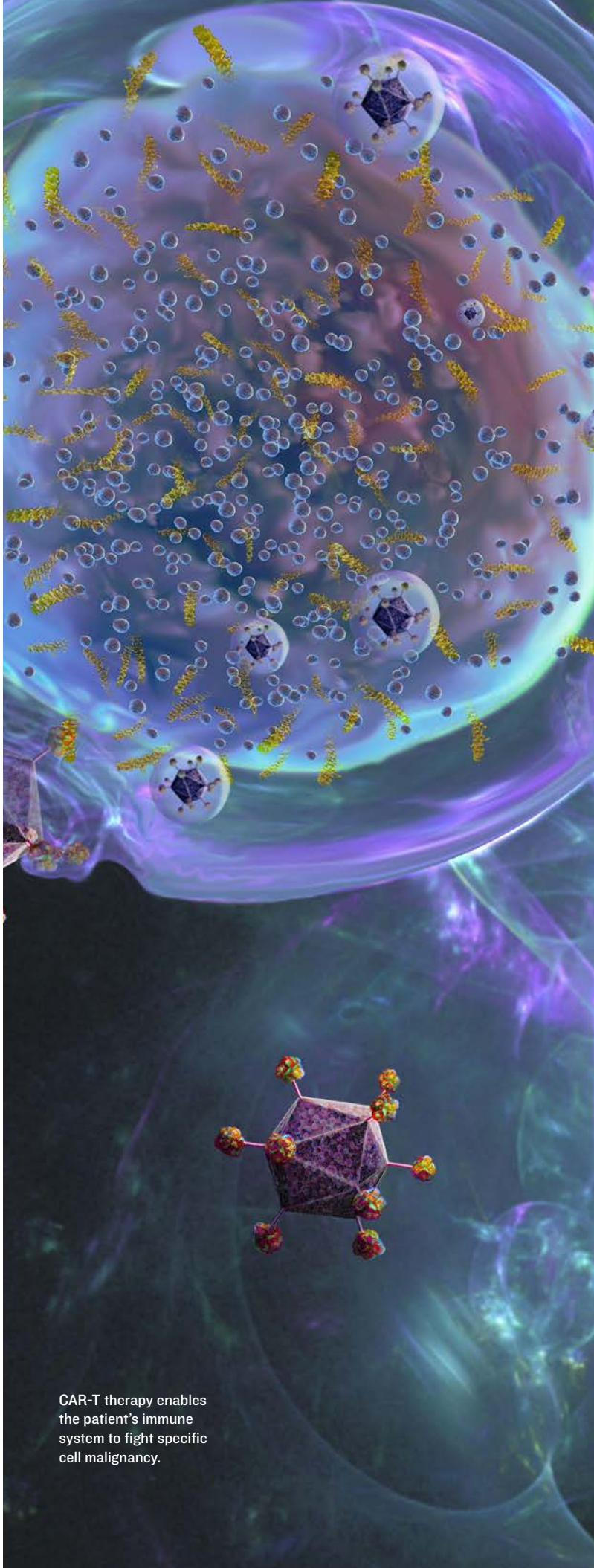
The growing role of cell and gene therapies

Extensive collaboration between various stakeholders in the field of cell and gene therapies should expand the reach of these advanced treatments for multiple diseases.

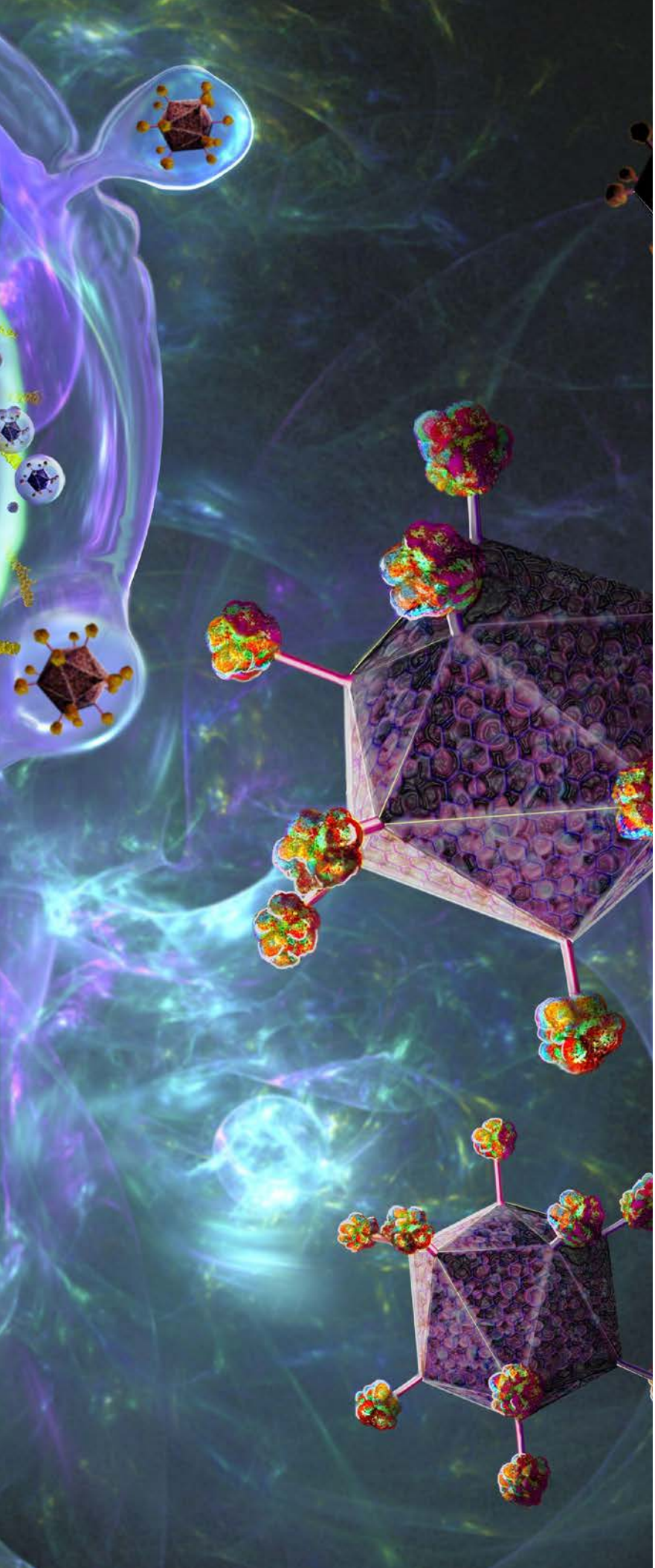
"We are in a dynamic environment in the cell and gene therapy space. The field is in the midst of groundbreaking innovations for the treatment of many diseases, and is benefitting more and more patients," says Miriam Fuchs, a global therapeutic area lead in Regulatory Affairs at the Swiss-based global healthcare company Novartis. "Revolutions leading to new therapies, and evolutions to further improve these therapies, are moving at an amazing speed."

Fuchs spent many years as an academic researcher in the field of cancer biology before joining the pharmaceutical industry. She has extensive experience in the field of haematology, and was involved in the development and registration of tisagenlecleucel, the first approved chimeric antigen receptor T-cell (CAR-T) therapy. The therapy is now available for the treatment of certain forms of B-cell malignancies including indications in paediatric and young adult B-cell acute lymphoblastic leukaemia and diffuse large B-cell lymphoma.

CAR-T therapy is an innovative, complex cell and gene therapy. Doctors collect T cells, a type of immune cell from a patient, which are reprogrammed using genetic modification to express a chimeric antigen receptor, directed against a cell surface protein on the target cancer cells to attack it. The modified cells are released in the body again. This precision, personalised therapy enables the patient's immune system to fight their specific B-cell malignancy.



CAR-T therapy enables the patient's immune system to fight specific cell malignancy.



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"Cell and gene therapies are an amazing mix of science and innovation," says Fuchs. "In the case of CAR-T therapy, every individual product has the potential to have a meaningful impact for its patient. This is very rewarding and extremely motivating for those of us working in this field." At Novartis's manufacturing sites for tisagenlecleucel, they maintain an illuminated 'wall of hope'; for every patient batch they manufacture, they turn on one additional light as a symbol of their commitment to patients.

Fuchs shared some of Novartis's key learnings from their journey in developing and commercializing tisagenlecleucel at the Riyadh summit. Their hope is to widen access to such therapies, alongside the continued efforts to refine and optimise each treatment.

"Our learnings may be valuable considerations for other developers in the advanced therapies field," says Fuchs. "Most importantly, there needs to be a strong emphasis for all stakeholders —developers (academia, biotech, pharma), regulators, payers, physicians and patients—to work closely together to enable key innovations to reach those in need."

Public education about all forms of cell and gene therapy is required to build trust in novel therapeutics, which can sometimes seem like science fiction to the general public.

"We must drive education forward, ensuring that accurate information presented in lay terms is available to all patients and to the wider public," says Fuchs. Describing the science behind new technologies and treatments clearly, and sharing success stories and case studies as well as the complexities of these innovations, will help the public better understand and accept these novel therapies. "As these therapies become reality for more and more people, awareness will be increased, helping to boost trust and enhance understanding of these therapies."

Miriam Fuchs, global therapeutic area lead in Regulatory Affairs at Novartis.