BR25293293 "Implementation of CAR-T Therapy Technology for Hematological Tumors into Practical Healthcare."

Program Implementation Period: 2024–2026

Relevance. The introduction of CAR-T therapy is highly demanded by patients with relapses after available therapies and their families, as well as by oncologists, who expect that CAR-T will enable treatment for 25% of patients with relapsed or refractory diseases.

How the Goal Will Be Achieved: Key Approaches: The implementation requires collaboration among oncologists for patient management, biotechnologists for manufacturing therapeutic cellular products, and transfusion specialists qualified to collect blood cells, as current regulations permit blood collection for therapeutic product manufacturing only in licensed institutions of the Blood Service.

The National Center for Biotechnology (NCB, Astana), in partnership with clinics, the National Scientific Oncology Center (NSOC, Astana), and the Scientific and Production Center for Transfusionology (SPCT, Astana), has completed preparatory work for the introduction of CAR-T therapy.

Other program participants include the S.D. Asfendiyarov Kazakh National Medical University (KazNMU, Almaty) and the National Holding “QazBioPharm” (Astana).

Currently, the introduction of unregistered and complex therapies such as CAR-T is only possible under hospital exemption rules, as established by Ministry of Health Order №240 for the application of Breakthrough Therapy Medicinal Products (BTMP) [1].

CAR-T will be implemented under hospital exemption rules by administering therapeutic cellular products to six NSOC patients based on clinical indications. Patients will be selected from those with relapsed disease following the last line of standard therapy, for whom CAR-T represents the final chance for remission, with the potential for long-term remission.

Research protocols have been developed and approved by local ethics committees at NCB and NSOC. The following activities are planned: Patients enrolled in the study will receive appropriate premedication. Initial blood cells (from patients) will be collected at SPCT. NCB will manufacture the therapeutic cellular products. Quality control (QC) of the cellular product will be conducted using the CAR-T standards specified by the European Medicines Agency (EMA) or methods used by Novartis. At NSOC, the cellular product will be administered to patients, who will be monitored. Study results will be analyzed to assess safety and efficacy.

Program Goal. The goal is to introduce CAR-T receptor technology in Kazakhstan and apply it to the treatment of hematological malignancies.

Expected Results

Upon program implementation, the following outcomes are expected:

1. Production of cellular products for CAR-T therapy.
2. Formation of a consortium with clinical bases for CAR-T therapy application in Kazakhstan.
3. Development of research protocols for hospital exemption-based CAR-T therapy, with mentorship from experienced CAR-T specialists. Formation of hospital councils for patient selection.
4. Approval for clinical use of CAR-T therapy under hospital exemption, supervised by a mentor.
5. Accreditation of a testing laboratory for CAR-T therapy cellular products according to ISO 17025 or ISO 9001 standards.
6. Development and implementation of manufacturing regulations for CAR-T therapy cellular products at NCB.
7. Clinical implementation of CAR-T therapy by treating up to three patients under hospital exemption in compliance with good practices.
8. Analysis of data on cellular products provided by the manufacturer, with results presented in a scientific article.

9. Analysis of clinical application results, including safety and efficacy evaluation. Preparation of a final report for the national regulator and publications. Presentation of results at medical and scientific congresses and conferences.

10. Publication of at least four articles and/or reviews in peer-reviewed scientific journals indexed in the Web of Science Core Collection Science Citation Index Expanded and/or with a CiteScore percentile of at least 35 in Scopus, as well as at least two articles and/or reviews in domestic or international journals recommended by the Ministry of Science and Education of Kazakhstan. Submission of one patent application based on program results.

Team Information

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