

Substantive assessment form for the application No.



CO-FUNDING APPLICATION SUBSTANTIVE ASSESSMENT FORM

Development of Polish (CAR/CAR-T) adoptive therapy with the use of genetically modified immunocompetent cells

COMPETITION NUMBER: ABM/2020/4

PROJECT TITLE:

APPLICANT:

DATE OF RECEIPT OF THE APPLICATION:

PERSON ASSESSING THE APPLICATION:

Substantive assessment form for the application No.

Criterion name	Maximum / minimum total number of points required in order for the project to be assessed positively	Number of points awarded
<u>Part A: Statutory criteria</u>		
<p>Criterion 1: Scientific value of the project (from 0 to 20 points) that includes:</p> <ul style="list-style-type: none"> • the objective of the research (accuracy of definition of the scientific problem, quality of presentation of the state-of-the-art - (state of the research) based on the current knowledge. This item shall first of all assess the main subject of the Project and it will result in registration on non-commercial clinical research (from 0 to 5 points); • correctness of selection of research methods and tools in the design of the clinical research, including the sample size, availability of patients, defining the research end points. Also the presented initial results that confirm validity of the proposed Project shall be assessed (from 0 to 5 points); • correctness of task planning and division of labour between Consortium members in the context of the intended objective of the Project. The way the tasks were planned within the Project and assigned to the Partners, 	20/10	

Substantive assessment form for the application No.

<p>transparency of the schedule of the planned works and consistency of those elements with the budget shall be assessed (0 pts to 5 pts)</p> <ul style="list-style-type: none"> the final result of the proposed Project, that has to be defined precisely. It shall be assessed whether the assumed results of the research work are feasible in the context of the assumed schedule and budget. Also description of identified and precisely defined (physical, technical, psychological, social, legal, regulatory or economic) risks connected to both the research programme as well as preparation and conducting the research shall be assessed (from 0 to 5 points). 		
<u>Statement of reasons:</u>		
<p>Criterion 2: Impact of the Project on the improvement of the health of citizens (a TOTAL of 40 points maximum), taking into account the need to:</p> <ol style="list-style-type: none"> save life and obtain a full recovery (from 0 to 10 points), save life and obtain health improvement (from 0 to 10 points), prevent premature death (from 0 to 10 points), improve the quality of life (from 0 to 10 points); 	40/20	
<u>Statement of reasons:</u>		

Substantive assessment form for the application No.

<p>Criterion 3: Innovative nature of the Project Within this criterion the following shall be assessed:</p> <ul style="list-style-type: none"> the main subject of the Project, namely development of CAR/CAR-T Therapy by the subjects that are based and operate in the Republic of Poland and testing it in a clinical trial (from 0 to 10 points), the subject connected to the research of the use of the CAR/CAR-T in new applications, such as treatment of solid tumours, autoimmune, degenerative, infectious diseases, etc. (from 0 to 10 points). 	20/10	
<p><u>Statement of reasons:</u></p>		
<p>Criterion 4: Expected economic outcomes (from 0 to 20 points) that include:</p> <ul style="list-style-type: none"> Demonstration and justification of economic benefits that result from the potential use of the outcomes of the Project in medicinal practice in relation to the current costs, including indirect costs for the health care and welfare system. Potential economic benefits resulting from the main subject of the Project, namely development of a CAR/CAR-T Therapy, as well as side subjects, namely possible application of therapies of this type in new indications, shall be assessed (from 0 to 10 points). The estimated cost of the CAR/CAR-T Therapy being developed versus realistically assessed savings to the budget the therapy may result in. The co-funding 	20/10	

Substantive assessment form for the application No.

<p>application shall include an analysis supported by a number of patients in Poland and Europe who could potentially benefit from the adoptive therapy, the costs of current treatment of the specific disease through the whole life of a patient, probable (but accurately estimated and documented) cost of one administration of the developed CAR/CAR-T Therapy (from 0 to 5 points).</p> <ul style="list-style-type: none"> • Proper identification and description of possible risks/threats/barriers that would pose considerable problems to reaching positive economic outcomes (from 0 to 5 points). 		
<u>Statement of reasons:</u>		
<p>Criterion 5: Possibility of using the outcomes of the Project in the healthcare system (from 0 to 20 points) that includes:</p> <ul style="list-style-type: none"> • A justification of the need for the outcomes of the Project by the healthcare sector - including defining disease unit/units and healthcare system problems connected to them, defining the target group (patients), presenting the number and quality of market data that are the basis for the justification (from 0 to 10 points); • Analysis of putting the outcomes of the Project to practice on the basis of market data (from 0 to 5 points); • Assessment of transfer of the know-how and new technologies that are the outcome of the Project to the 	20/10	

Substantive assessment form for the application No.

healthcare system based on an analysis of similar therapies that include ATMP medicinal products (from 0 to 5 points)		
<u>Statement of reasons:</u>		
Criterion 6: Possession of material and human resources by the Consortium members, required to perform the project (from 0 to 10 points).	10/5	
<u>Statement of reasons:</u>		
TOTAL points awarded in part A	130/65	

Substantive assessment form for the application No.

Part B: Specific criteria	
<p>Criterion 1:</p> <p>Is the co-funding application submitted by a Consortium that satisfies the requirements defined in section 1.5 and 1.5.1, namely:</p> <ul style="list-style-type: none"> • The Consortium Leader is a public higher education institution that offers courses at medical faculty, which is a founder of a healthcare facility that is a part of the Consortium, and the research institute mentioned in art. 3 of the Act of 30 April 2010 on research institutes. • Consortium members (other than the Leader): <ol style="list-style-type: none"> 1) at least one medical entity responsible for the production in Poland of CAR/CAR-T cell products (and possibly administration thereof to patients), whereas the entity may participate in research activity under the project, 2) at least 3 (three) medical entities with documented experience in the administration of hematopoietic cells which will perform procedures related to the administration of CAR/CAR-T cells, exercising comprehensive care of patients in which a therapy based on CAR technology has been applied. These entities may participate in research works under this Project, 	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY

Substantive assessment form for the application No.

<p>3) other entity or entities mentioned in Article 17 of the Act on the Medical Research Agency, with documented experience in immunology, oncology, molecular biology, pre-clinical trials</p> <p>- each of them being an entity of the public finance sector or a research institute, in terms of the act of 30 April 2010 on research institutes? - SATISFIES / DOES NOT SATISFY.</p>	
Statement of reasons:	
<p>Criterion 2:</p> <p>Each of the healthcare entities forming part of the consortium that files the co-funding application must have a team, infrastructure and experience in performance of transplants of haemopoietic cells (at least 50 transplants of haemopoietic cells a year in adult patients or 15 transplants of haemopoietic cells a year in paediatric patients).</p>	<p style="text-align: center;"><input type="checkbox"/> SATISFIES</p> <hr/> <p style="text-align: center;"><input type="checkbox"/> DOES NOT SATISFY</p>
Statement of reasons:	
<p>Criterion 3:</p> <p>Each Consortium member may only submit one co-funding application as part of the call, whether as the Leader or as a partner of the Consortium.</p>	<p style="text-align: center;"><input type="checkbox"/> SATISFIES</p> <hr/> <p style="text-align: center;"><input type="checkbox"/> DOES NOT SATISFY</p>

Substantive assessment form for the application No.

Statement of reasons:	
Criterion 4: The project envisages that the entire technological process connected with CAR-T cells production will take place within the territory of Poland. Production of the vectors necessary to prepare CAR/CAR-T cells outside the territory of Poland is possible.	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY
Statement of reasons:	
Criterion 5: The Project envisages that the intellectual property rights to the developed CAR/CAR/CAR-T Therapy remain the property (non-transferrable to any extent) of the Consortium Leader.	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY
Statement of reasons:	
Criterion 6: The project envisages that registration of the clinical trial related to CAR-T Therapy developed within the framework of the Project will take place no later than 3 years after commencement of the Project. Clinical trial shall be a multicentre trial.	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY

Substantive assessment form for the application No.

Statement of reasons:	
<p>Criterion 7:</p> <p>The project envisages that at least 150 persons who meet the medical criteria and are entitled to healthcare under the public healthcare system within the territory of the Republic of Poland will be administered the CAR-T Therapy. The 150 persons include:</p> <p>a) the patients that will receive a CAR/CAR-T Therapy that is commercially available, financed from the Project budget (this type of administration is possible for the first 3 years of the Project) and</p> <p>b) the patients enrolled to a non-commercial clinical trial started within the Project, who will receive the developed CAR/CAR-T Therapy</p>	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY
Statement of reasons:	

Substantive assessment form for the application No.

<p>Criterion 8:</p> <p>The project provides at least for the following activities:</p> <ol style="list-style-type: none"> 1) Develop and optimise the use and improve the safety of CAR/CAR-T cell Therapy in the treatment of blood cancers in Poland by developing the infrastructure and a team capable of producing and administering CAR/CAR-T cells. 2) Obtain appropriate permits and certificates for the production of CAR/CAR-T cells and their use in the treatment of patients (development of the production method). 3) Prepare the trial protocol and register the trial. 4) Perform a clinical trial, including create an electronic medical database to measure the clinical effect and safety of the CAR/CAR-T Therapy in clinical practice. 5) Conduct research activity which will enable the use of CAR/CAR-T Therapy for other indications (e.g. solid tumours, infectious diseases, autoimmune or degenerative diseases) and innovation in CAR technology. 6) Develop and implement a programme for training of healthcare professionals involved in treatment of patients with the use of CAR/CAR-T cells. 7) Establish a Polish network of healthcare facilities able to manage the CAR/CAR-T technology. 	<p style="text-align: center;"><input type="checkbox"/> SATISFIES</p>
	<p style="text-align: center;"><input type="checkbox"/> DOES NOT SATISFY</p>

Substantive assessment form for the application No.

8) Manage and administer the Project.	
Statement of reasons:	
Criterion 9: The process developed within the Polish CAR/CAR-T Therapy should lead to maximum reduction of time between apheresis and delivery and administration to the patient should not be longer than 4 weeks.	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY
Statement of reasons:	
Criterion 10: The project includes a description of the quality control, transportation and cell storage processes, which ensures a high quality and safety of the CAR/CAR-T Therapy for the patient.	<input type="checkbox"/> SATISFIES
	<input type="checkbox"/> DOES NOT SATISFY

Substantive assessment form for the application No.

<u>Part C: Bonus criteria</u>	
<p>Criterion 1:</p> <p>The person indicated as the Principal investigator has a proven track record of scientific achievements (at least 2 experimental publications - as the main author, published over the past 5 years in journals featured on the List of scientific journals and reviewed materials from international conferences with assigned number of points, published by the Ministry of Science and Higher Education, pursuant to art. 267, paragraph 3 of the act of 20 July 2018, Law on Higher Education and Science (Journal of Laws, item 1668, as amended), that is valid on the day the co-funding application is being filed – 10 points for each publication (max. 20),</p> <p>Score weight: 10 (maximum 20)</p>	<input type="checkbox"/> SATISFIES – points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points
Statement of reasons:	
<p>Criterion 2:</p> <p>The Applicant has a proven track record in international scientific cooperation (joint projects/publications) aimed at conducting research on cancer immunotherapy.</p> <p>Score weight: 10</p>	<input type="checkbox"/> SATISFIES – 10 points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points

Substantive assessment form for the application No.

Statement of reasons:	
Criterion 3: The Applicant has proven track record of experience in conducting clinical trials that test Advanced Therapy Medicinal Products ATMP. Score weight: 10	<input type="checkbox"/> SATISFIES – 10 points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points
Statement of reasons:	
Criterion 4: The project includes development and carrying out research in at least one additional indication, beside the obligatory for the Project treatment of blood cancers. For each additional therapeutic indication the Applicant shall receive 10 points. Score weight: 10 (maximum 20)	<input type="checkbox"/> SATISFIES – points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points
Statement of reasons:	

Substantive assessment form for the application No.

Criterion 5: The number of patients covered by the project is greater than the number specified in criterion 7 – for every 10 additional patients – 10 points (max. 30 points) Score weight: 10 (maximum 30)	<input type="checkbox"/> SATISFIES – points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points
Statement of reasons:	
Criterion 6: The project envisages creation of a “Core Facility”/research facilities (incubator) within the Consortium for Polish scientists/international teams which include Polish scientists for the purpose of implementation of research projects aimed at the development of adoptive therapies. Score weight: 20	<input type="checkbox"/> SATISFIES – 20 points
	<input type="checkbox"/> DOES NOT SATISFY – 0 points
Statement of reasons:	
Criterion 7: Spending planned for infrastructure is less than 25% of total costs of	<input type="checkbox"/> SATISFIES – points

Substantive assessment form for the application No.

<p>the Project - for decreasing this category of spending by each percentage point, the Project obtains 2 points.</p> <p>Score weight: 2 (maximum 20)</p>	<p><input type="checkbox"/> DOES NOT SATISFY – 0 points</p>
<p>Statement of reasons:</p>	
<p>TOTAL points awarded in part C</p>	

Substantive assessment form for the application No.

<u>Part D: Summary</u>	
1. Number of points awarded for statutory criteria (part A)	
2. The project meets all the specific criteria (part B)	<input type="checkbox"/> YES
	<input type="checkbox"/> NO
3. Number of points awarded for bonus criteria (part C)	
Total points awarded	
The project may be co-funded	<input type="checkbox"/> YES
	<input type="checkbox"/> NO

Substantive assessment form for the application No.

Statement of reasons:
