

PharmaCell BV – PhC

H2020 Calls Focus

SC1-PM-11 Clinical Regenerative Medicine. April 2016 (Single stage)

<https://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/topics/3053-sc1-pm-11-2016-2017.html>

SC1-PM-08 Clinical Research in Rare Diseases. October 2016 (2 stages)

<https://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/topics/2436-sc1-pm-08-2017.html>

SMEInst-05-2016-2017 SME in Biotech. Starting February 2016 (multiple cut-off days)

<https://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/topics/6117-smeinst-05-2016-2017.html>

participation in other calls (as far as the focus is on Cell Therapy and Ex Vivo Gene Therapy will be evaluated on a case by case basis.

H2020 Consortia possible Activities

Within the panorama of the above mentioned calls PharmaCell can contribute with the following activities:

- Cell and Ex Vivo Gene Therapy manufacturing/release for clinical Phase I-III studies under GMP (both manual and automated platforms)
- Process and Assay qualification/validation up to the level required for the current and future phases
- Process, Assays, and Product Development for following clinical/commercial phases
- Consulting on Good Manufacturing Practices, Advanced Therapy Medicinal Products, development, manufacturing, and distribution.

General description of the organisation and the departments

PharmaCell (PhC) is a leading Contract Manufacturing Organization for Cellular Therapies and Regenerative Medicine in Europe located in Maastricht, Netherlands. PhC operates two state-of-the-art cGMP-licensed facilities located centrally in Europe, and supports Phase I to Phase III clinical trials. Since 2006 (first GMP license granted) PhC has gained an extensive track record in the development and daily management of biomanufacturing processes in cell therapy field. Next to clinical trials manufacturing, PhC is also the manufacturer of two (out of three) EMA-approved cell therapy products for the European market.

The company structure is divided in six departments: Production, Quality Control, Quality Engineering, Quality Assurance, Process Development & Research, and Project Management, for a total of 60+ people. For each project a cross-departmental core-team is created to follow the product through its entire life-cycle within the company (Technology Transfer, Non-Clinical Implementation, Clinical Production, and Commercial Production).

PhC is managing two state-of-the-art facilities (1400+3600 m²) with two licensed clean-rooms (250+750 m²) for cell therapy production under cGMP for both clinical and commercial purposes. PhC has a total of five class B rooms equipped with a total of 11 Class A Laminar Air Flow Cabinets (comprehensive of a suite for handling of virus positive material). The clean rooms comprehend class C (8) and class D (8) rooms used for closed system bioprocessing, equipment/material preparation, incubation and Quality Control. Additionally the facilities have cryo-storage capacities, warehouses, and dedicated additional Quality Control and Process Development labs, all fully controlled under our cGMP compliant quality system. Our Production, Quality Control, and Process Development suites contain equipment such as

LAFC, incubators, stirred bioreactors, flow cytometers (CANTO II), inverted microscopes, qPCR devices, spectrophotometers, automated cell counting devices, and Biosafety assay equipment (e.g. endotoxin). A non-comprehensive list of activities performed within the company: GMP-compliant open & closed cell manufacturing (autologous and allogeneic); GMP-compliant safety, identity testing, flow cytometric analysis, functional assays; in-house endotoxin, myco-alert, bioburden, FACS analysis, ELISA testing; real-time release by in house Qualified Persons; GMP compliant validation & maintenance of equipment, utilities and building. PhC has also an extensive experience in Technology Transfer both for manufacturing processes and quality control assays. Finally the company has a dedicated Process Development and Research Department active in: i) process, product, and assay development; ii) scientific and technical support throughout the entire product life-cycle; iii) management of publicly funded research projects; iv) strategic technology evaluation, testing and implementation.

Description of the profile of key personnel

Nasser Sadr, PhD (Biomedical Engineer); Function: Manager Process Development and Research Department; He has 10 years scientific and technical experience in ATMP, working both in academia and industry on manufacturing processes and quality control assays (development and validation). NS has published 11 papers on international peer reviewed journal, 1 book chapter, and holds 2 provisional patent applications (furthermore: 23 abstracts at international conferences, 1 Young Investigator Award, 1 First Prize at National Innovation Business Plan Competition).

Ruben Heinen, MSc (Biotechnology); Function: Head Production Department. He has 10 years operational experience in pharmaceutical (GMP) manufacturing, of which 4 years in ATMP manufacturing, with a focus on Technology Transfer process for cell-based therapies in accordance with GMP regulations.

Behnaz Shokouhi, PhD (Cell Biologist); Function: Manager Technology Transfer and member of the Production Department. She has 9 years scientific and operational experience in ATMP, with a focus on Technology Transfer process for cell-based therapies in accordance with GMP regulations. BS has published 7 papers on international peer reviewed journal, 1 book chapter, and (furthermore: 6 abstracts at international conferences, 1 Best Poster Prize, and the Wilhelm Borschers Medal of RWTH for Distinguished Ph.D.

Hera Lichtenbeld, PhD (Cell Biologist); Function: Project Manager and member of the Project Management Department. She has over 20 years of scientific and industry experience. HL published 13 papers in international peer reviewed journals, 1 book chapter, and holds 1 Patent (US 6,368,858). She also received several travel grants and a research fellowship from the Natl.Cancer Inst., Bethesda, USA.

Alexander Vos, MBA (Pharmacy and Pharmacology); Function: CEO; He has 20+ years of leadership experience in the pharma and biotech industry.

List of 5 relevant publications/products/services or achievements

Relevant manufactured products:

- PhC has over the years prepared clinical material for 15+ preclinical and clinical studies (Phase I to Phase III) significantly contributing to the translation of ATMP from bench to bedside.
- PhC has also been selected for the commercial production of the first Authorized Cell-based regenerative product (ChondroCelect, by Tigenix) and the first Cell-based Cancer Vaccine in the EU (Provenge, by Dendreon).

Relevant publications:

- Piola M, Soncini M, Cantini M, **Sadr N**, Ferrario G, Fiore GB *Design And Functional Testing Of A Multichamber Perfusion Platform For Three-Dimensional Scaffolds*. Scientific World Journal. 2013 doi: 10.1155/2013/123974.
- **Sadr N**, Pippenger BE, Scherberich A, Wendt D, Mantero S, Martin I, Papadimitropoulos A. *Enhancing The Biological Performance Of Synthetic Polymeric Materials By Decoration With Engineered, Decellularized Extracellular Matrix*. Biomaterials 2012 33(20):5085-93
- **Sadr N**, Zhu M, Osaki T, Kakegawa T, Yang Y, Moretti M, Fukuda J, Khademhosseini A *Sam-Based Cell Transfer To Photopatterned Hydrogels For Microengineering Vascular-Like Structures* Biomaterials 2011 32(30):7479-90
- Piccinini E, **Sadr N**, Martin I *Ceramic Materials Lead To Underestimated DNA Quantifications: A Method For Reliable Measurements*. Eur Cell Mater 2010 20:38-44.
- Mantero S, **Sadr N**, Riboldi SA, Lorenzoni S, Monetevecchi FM, *A new electro-mechanical bioreactor for soft tissue engineering*. J Appl Biomater Biomech. 2007 5(2):107-16.

Relevant Achievements:

- Only CMO worldwide to have manufacture two cell therapies with approved market authorization.

List of 5 relevant projects or activities relevant to the call content.

PhC is or has been involved in numerous privately funded product development and clinical trial projects. Additionally the company has participated as a partner in the following publicly funded projects:

- i) FP7- Health/602366 Health.2013.4.1-2 **AGORA** ATMP GMP Open Access Research Alliance.
- ii) FP7-Health/ 304914- Health.2012.1.4-2 **BALANCE**: Development of a bioartificial liver therapy in acute liver failure;
- iii) BioMedical Materials Program Funding. **BioKid**: Living membranes for an Intradialytic Biological Kidney Support Device;
- iv) BioMedical Materials Program Funding. **SMARTCARE**: SMART microtissues for CArdiac Repair;
- v) Funding from Province of Limburg 2006-035 **Intact**: Regenerative Medicine using autologous stem cell technologies for treatment of cardiovascular diseases.

A H2020 projects PHC-16 “Tools and Technologies for Advanced Therapies” is now in Grant Agreement Signature phase.

Any other relevant information.

- PhC is represented in a number of ATMP relevant associations such as: International Society for Cellular Therapy; Alliance for Regenerative Medicine actively participating in the Scientific and Technical committees. PhC is also involved in standard definitions as a member of the ISO Technical Committee 276 (WG3 and 4).
- PhC as part of effort in maintaining and expanding the GMP licenses is in continuous contact with the competent authorities (e.g. Inspectie voor de Gezondheidszorg) from which the company is regularly inspected.

To further discuss opportunities please contact Nasser Sadr (n.sadr@pharmacell.nl) or Soenke Brunswieck (s.brunswieck@pharmacell.nl) or call +31433509910.