CTA approval for potentially pivotal Heartcel™ Phase IIb trial

- Adult heart failure trial to complete in 2020 with potential market entry in 2021
- Trial to be conducted at Imperial College London's Royal Brompton, UK

Stratford-upon-Avon, UK, 8 January 2018 – Celixir, a private company discovering and developing life-saving regenerative medicines, today announced it has received formal clinical trial application (CTA) approval from the Medicines and Healthcare Products Regulatory Agency to initiate a Phase IIb human clinical trial with its Heartcel™ medicine for the treatment of moderate to severe adult heart failure. Heart failure affects over 25 million adults worldwide with current medicines not slowing disease progression or prolonging life. Up to 45% of patients in hospital with heart failure die within one year of admission[1].

Heartcel™, Celixir's lead investigational cardiac regenerative medicine, is a tissue engineered medicine comprising allogeneic (off-the-shelf) immunomodulatory progenitor (iMP) cells and is designated as an advanced therapeutic medicinal product (ATMP) by the European Medicines Agency. iMP cells are a novel cell type engineered to reduce heart scarring and regenerate the heart in patients with heart failure. iMP cells are currently delivered during coronary artery bypass graft but catheter delivery is in development to expand the indication to all heart failure and cardiomyopathy patients.

The Heartcel™ Phase II results presented at the 2016 International Society of Stem Cell Research Annual Conference showed the study met all the endpoints with statistical and clinically significant results. These 12-month endpoints included 100% MACE-free survival (major adverse cardiac event); 30% average improvement in left ventricular ejection fraction, 40% reduction in left ventricle scar size and 50% improved quality of life.

At the four year follow-up, MACE-free survival remains 100% and imaging analysis evidenced scar reduction and heart regeneration in 70% of patients. Replicating these results in larger global trials with, could make Heartcel™ eligible for conditional marketing authorisation in Europe and FDA accelerated approval in the US.

Professor Stephen Westaby, CMO, former Consultant Heart Surgeon, John Radcliffe Hospital, Oxford said: *"Heart failure is still a major cause of death affecting millions of patients worldwide. Current therapies do not reduce chronic myocardial (heart) scarring, diminish disease progression or prolong life. Thus, the initial Heartcel trial results are very exciting, showing the first evidence in humans of disease reversal, scar reduction and heart regeneration.*

"We are assembling a world-class team at Imperial College, in the US and in Asia to accelerate patients' access to this potential breakthrough in treatment of heart failure."

Ajan Reginald, Chief Executive Officer of Celixir, commented: *"All credit to our exceptional Celixir team for an outstanding year in 2017: completing an institutional funding round, gaining GMP manufacturing approval, completing the FDA pre-IND process, and gaining CTA approval for the*

potential pivotal trial in Europe. We're delighted to be working with the leading academics at Imperial College London, Harvard and MIT to accelerate Heartcel's development. We plan to maintain this momentum in 2018 with significant milestones in the US and Asia."

[1] Heart failure, preventing disease and death worldwide. European Society of Cardiology (Ponikowski et al).

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About Celixir

Celixir (formerly known as Cell Therapy Ltd.) is a private British regenerative medicine company that discovers and develops life-saving and life-altering regenerative medicines for patients with the greatest medical need. Celixir, founded in 2009, is made up of a world class team of scientists and biopharmaceutical executives, led by Nobel Laureate Professor Sir Martin Evans and former Roche Global Head of Emerging Technologies, Ajan Reginald. Celixir's unique platform technology allows them to adopt an 'off-the-shelf' approach to deliver regenerative medicines to patients.