



GMP Cell and Gene Therapy Manufacturing

ADVANCED CELL THERAPY LABORATORY

Cell and gene therapy (CGT) is transforming health care.

CGT is personalized medicine revolutionizing the way we treat disease and has the potential to prevent, treat or cure a range of medical conditions. CGT products are inherently complex, have shorter shelf life, and are more variable. Specialized technical knowledge in process and analytical development and Good Manufacturing Practices (GMP) facilities are essential to bring these therapies into clinic. GMP manufacturing process ranges in complexity and faces multiple challenges. As the number of CGT products entering clinical trials has accelerated, GMP manufacturing is in high demand and risks becoming a bottleneck to treat patients.

The UC San Diego Advanced Cell Therapy Laboratory (ACTL) is supporting CGT entrepreneurs. Since 2018, the ACTL GMP facility, under a license from the California Department of Public Health, has done GMP CGT manufacturing for clinical trials run by UC San Diego researchers and regional partners. ACTL is led by Dan Kaufman, MD, PhD, as the scientific/medical director and Holly Young as the facility director.

ACTL clients include a mix of UC San Diego investigators and outside companies. The services provided by ACTL include 1) consultation and advising about CGT projects, 2) process development work to translate research lab studies to GMP manufacturing to enable IND submissions, 3) GMP cell therapy manufacturing for clinical trials, 4) final formulation and fill services for clinical trials and 5) long-term, monitored cryo storage of cell therapy products.

GMP facility

UC San Diego established a 2,000-sq.-ft. ACTL GMP facility in 2016 to support early stage clinical trials

ACTL must expand to meet demand

Grow: A new, larger GMP facility will unite our staff with needed on-site equipment and warehouse space; enable researchers to develop new, more efficient processes; and increase access to our work and services to enhance outreach for new and existing partners.

Innovate: With growing demand for novel approaches, we are innovating our manufacturing processes to comply with regulatory guidance from EU and U.S. agencies while working with clients to expedite their product development.

Location: A permanent facility will build upon our reputation for excellence and ensure our local and national partners can continue to seek our world-class research and services.

At the University of California San Diego, visionary partners help us unleash a diverse community of doers: those who imagine unexpected answers that can transform humanity for the better. Together, we foster bold scholars, researchers, healers, entrepreneurs and creators. Because here, breaking new ground is the norm — **and people are the point.**



Advanced Cell Therapy Laboratory Priorities

Support for ACTL will be vital for the burgeoning field of CGT in San Diego.

Rapid growth of biotechnology companies in the San Diego region has increased the need for GMP CGT manufacturing especially for early stage trials. The development of public-private partnerships is essential for the next phase of ACTL.

With such a partnership ACTL would aim for construction of

- » New 40- to 60,000-square-foot facility suitable for early stage manufacturing.
- » Eight to 15 clean rooms, depending on the scope of the work to be done.
- » Space for genetic vector production. Given the nationwide shortage of space for production of GMP vectors (e.g., retrovirus, lentivirus, adeno-associated virus), we need to expand these facilities to avoid lengthy delays in vector production, which inhibits clinical cell manufacturing and clinical trials for products such as CAR-T cells and other engineered products.
- » Suitable lab space for process development (non-GMP research and development work), quality control (QC) lab space, warehousing, and liquid nitrogen storage.
- » Space for education and training a future skilled workforce in GMP, additional personnel and research activities, including office and meeting spaces for vibrant exchange of ideas.

PARTNER BENEFITS

- » Access to novel technologies related to cell and gene therapy manufacturing at UC San Diego, such as manufacturing technology from the Department of Bioengineering.
- » Access to novel cell and gene therapies being developed within Moores Cancer Center at UC San Diego Health and by other researchers on campus.
- » Opportunity to meet with startup companies working in CGT.
- » Invest in the exciting future of top talent by supporting internships for undergraduates, graduate students and postdocs.

[Learn more at actl.ucsd.edu.](http://actl.ucsd.edu)

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