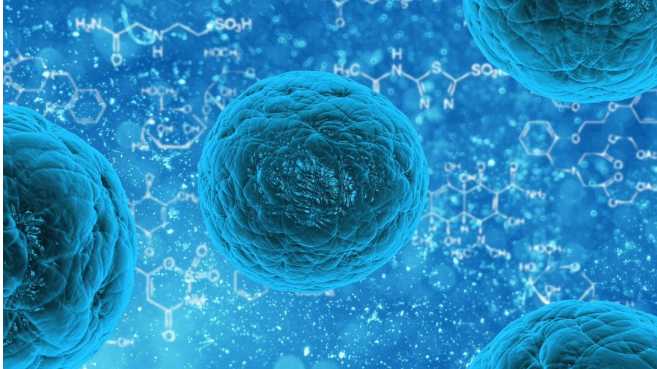


Largest source of AATD stem cells collected

2 July 2020



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Researchers from the Center for Regenerative Medicine (CReM) at Boston University and Boston Medical Center (BMC) have assembled the largest repository of patient derived stem cells (iPSCs) from patients with alpha-1 antitrypsin deficiency (AATD).

"Our goal is to get these cells out to other researchers so that they will use them to ultimately help patients with AATD. To do that, we needed to do everything possible to make the cells easy to get and easy to use," explained corresponding author Andrew A. Wilson, MD, associate professor of medicine at Boston University School of Medicine (BUSM).

AATD is a genetic disease that can affect the liver or lung. Patients lack the alpha-1 protein (made by the liver) which is designed to protect tissues in the body from being attacked by its own enzymes. iPSCs are increasingly being used to study [genetic diseases](#). They can be induced in the lab to turn into ("differentiate") [cell types](#) affected in disease, in this case lung and [liver cells](#). Once that happens, they can be used to understand how the disease works and potentially then use either the cells or that understanding to find new treatment approaches.

The researchers collected blood or skin samples from AATD patients over a period of approximately 13 years. In addition, they assembled clinical data associated with each patient/sample. They then performed [genetic analysis](#) to determine what genes were turned on for each patient iPSC line at baseline and then after becoming lung or liver cells. They also compared the iPSC-derived liver cells to liver cells obtained by biopsy from healthy individuals. Finally, they created an on-line resource to help disseminate these cells.

According to the researchers, this is important work for the AATD community as well as the general public. "AATD is the most common inherited, monogenic lung disease in Caucasians- about the same prevalence in the population as cystic fibrosis, just less well known. This study highlights the potential value of our open-access repository (which extends beyond AATD)," added Wilson, a pulmonary and critical care physician and director of the Alpha-1 Center at BMC.

CReM researchers have pioneered the concept of "Open-Source Biology" by making their [scientific results](#) and the tools used to generate them as easily available as possible as quickly as possible to the greatest number of investigators possible.

These findings appear online in the journal *Stem Cell Reports*.

Provided by Boston University School of Medicine

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