

THE DISTILLERY

This week in therapeutics

| Indication | Target/marker/pathway | Summary | Licensing status | Publication and contact information |
|--------------------|-----------------------|---|--|---|
| Infectious disease | | | | |
| HIV/AIDS | Caspase-1 (CASP1) | Human tissue culture studies suggest CASP1 inhibitors could help treat HIV infection. In cultures of normal human lymphoid tissue, HIV predominantly infected quiescent CD4* T cells, upregulated CASP1 and led to CASP1- activated pyroptosis, which was not observed in HIV-infected, activated CD4* T cells. Also in the HIV-infected issue cultures, <i>CASP1</i> -targeting shRNA or the small molecule CASP1 inhibitor VX-765 decreased CASP1 levels and the number of pyroptotic CD4* T cells compared with scrambled shRNA or no treatment. Ongoing work in collaboration with Vertex Pharmaceuticals Inc. may include a Phase II trial of the company's VX-765 in combination with antiretroviral therapies in patients with HIV infection. VX-765 is in Phase II testing to treat epilepsy. | Patent application filed by the Gladstone Institutes; licensing status undisclosed | Doitsh, G. <i>et al. Nature</i> ; published online Dec. 19, 2013; doi:10.1038/nature12940 Contact: Warner C. Greene, Gladstone Institute of Virology and Immunology, San Francisco, Calif. e-mail: wgreene@gladstone.ucsf.edu |

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