WILLIAM & MARY
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WM-1508: TREATMENT FOR FRONTOTEMPORAL DISORDER AND ALZHEIMER'S DISEASE

This is a 505(b)(2) drug opportunity to treat the rare disease Frontotemporal Dementia, and also Alzheimer's Disease.

Technology Background. D-cycloserine ("DCS", an old off-patent, rarely used drug to treat tuberculosis approved only as a 250 mg capsule) is an NMDA receptor partial agonist that enhances neuroplasticity and facilitates certain kinds of learning. Recent research from the University of Alabama-Birmingham showed potential efficacy for DCS in a rat model of frontotemporal dementia. DCS has shown significant efficacy (comparable to donepezil) for Alzheimer's in some smaller human clinical trials, but a 400-patient study conducted by Searle/Pfizer showed no benefits. This result is not surprising to us, as tolerance to DCS starts to develop within one day. Therefore, long-term daily administration of DCS is unlikely to be effective. Moreover, timing of administration can be important.

Intellectual Property. We have pending patent applications covering novel dosing strategies for DCS that do not induce tolerance, and also provide additional advantages.

Safety. While DCS has significant CNS side effects when dosed at 1,000 mg (four 250 mg pills) per day for tuberculosis, the side effects under our dosing strategy (a much lower dose given much less frequently) are almost non-existent. The drug will be extremely safe.

Manufacturing. US sales of DCS are miniscule, and the commercial drug (Seromycin) is owned by the Purdue University Research Foundation via a gift several years ago from Eli Lilly. The API is readily available, and significant CMC issues are unlikely. Note that there are no generic products; instead, there is only the off-patent Seromycin, and it is only approved as a higher dose capsule.

Clinical Development. We will need to perform a bioavailability study comparing our dosage to Seromycin. Non-clinical studies are unlikely to be required prior to IND submission, and only modest non-clinical studies would be required prior to submission of an NDA.

With less than $10M, we believe that this asset could be poised to commence pivotal Phase III clinical trials for FTD and Phase IIb trials for Alzheimer's Disease.

Market. There are about 60,000 cases of Frontotemporal Dementia in the US, and no approved drugs for that orphan condition. Obviously, the market for Alzheimer's Disease is much larger.

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