

# Biology Seminar

Biology Graduate Student Association, Department of Biological Sciences, and California Institute for Regenerative Medicine -- Fri. 2/3 @ 4pm in SCI B 133

**Pooling knowledge world-wide through rare disease patient family engagement can accelerate research to clinical impact, ADCY5 - related Dvskinesia as a case study**



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For rare disease patient communities, the challenge of low numbers of patients and islands of small data sets representing rare disease patient individuals compounded by the lack of economic incentives for academia and industry to work on rare disease programs contributes to little hope for undiagnosed/rare patient communities to find viable therapeutic solutions. An approach that emphasizes empowerment of the patient/community as equal stakeholders, pooling and democratizing data, and alignment of incentives through value driven collaborations can overcome the obstacles hindering progress in rare disease research and accelerate identification of clinical solutions. A scalable framework demonstrated by working with the ADCY5 patient community will be presented. It utilizes international patient engagement, data sharing, access to research tools such as patient derived stem cells that can leapfrog understanding of discovery research to clinical impact. The pilot ADCY5 program provides hope to not only accelerate finding more immediate therapeutic solutions for ADCY5 patients but also to provide a framework to leverage for other rare disease patient groups of similar biology and/or small patient communities where there are limited resources and research ongoing.

Biological Sciences Seminar Series venue is wheelchair-accessible. Persons who wish to request disability-related accommodations, including sign-language interpreters, should contact the Department of Biological Sciences, c/o peb112@humboldt.edu. Please request accommodations at least two days prior to the event. Some attendees may be sensitive to various scents from perfumes, shampoos, soaps, etc. Although optional, please consider refraining from wearing scented products to this event.