

Advancing the Arizona State University Knowledge Enterprise

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Selective Mitochondrial Imaging and Therapeutic Targeting

It is becoming increasingly apparent that mitochondrial dysfunction plays a critical role in numerous human diseases. Yet, there are no approved drugs designed to directly target mitochondria. To develop new therapeutics that target mitochondria, ideally, a mitochondrial targeting moiety would be utilized. Although several functionalities have been identified that localize to the mitochondria, these must typically be incorporated permanently within the structure of the potential therapeutic agent, complicating the design of the active agent.

Researchers at the Biodesign Institute of Arizona State University have developed novel caged probes for mitochondrial imaging and therapeutic targeting. Upon entering cellular mitochondria, these probes are activated by a mitochondrial enzyme and become fluorescent. This mitochondrial-specific enzymatic activation can be exploited to use the probes for both selective imaging and mitochondrial therapeutic targeting. These probes can thus be used for mitochondrial imaging and for selective delivery of prodrug forms of therapeutic agents to the mitochondria of diseased cells.

These novel probes demonstrate a new mechanism for mitochondrial imaging and targeted delivery, and can enable important advances in our quest for mitochondrial medicines.

Potential Applications

- Mitochondrial imaging
- Mitochondrial therapeutic targeting

o Hyperproliferative diseases, i.e. cancers

o Friedreich's ataxia

o Leber's Hereditary Optic Neuropathy

o Kearns-Sayre Syndrome

o MELAS (Mitochondrial Encephalomyopathy with Lactic Acidosis and Strole-like Episodes)

o Leigh's Syndrome

o Lou Gehrig's disease

o Diseases with significant mitochondrial component: PD, AD, Obesity, etc.

Benefits and Advantages

• This is the first mitochondria-specific activation of imaging and therapeutic agents

• Could be utilized as a new platform to develop novel therapeutic strategies for mitochondrial diseases or diseases characterized by mitochondrial dysfunction

- Can work with numerous types of imaging modalities
- Can work with many classes of therapeutic agents

For more information about the inventor(s) and their research, please see \underline{Dr} . Hecht's directory webpage