

ISIS Pharma - Picture Beyond KYNAMRO - Targeting Other Niche Indications

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Abstracts

Approval of KYNAMRO (mipomersen, apoB synthesis inhibitor, homozygous – HoFH; PhIII for heterozygous familial hypercholesterolemia –HeFH, partnered with Sanofi) in US and discounted pricing has removed the overhang of uncertainty. Upside from the New Orphan Indications with Global Partners (GSK, BIIB, AZN in 2012) and maturing pipeline remains. Successful systemic delivery of more drugs to the target organs other than liver is the acid test that all antisense/ RNAi focused companies have to pass. ISIS has a robust cash position, valuable IP assets, and success of a few drugs in the current pipeline could accelerate the transformation from a discovery to a pharma company and help it to grow beyond KYNAMRO.

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