Evaluate Vantage

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Ionis passes important efficacy hurdle

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So far, so good with the strong topline phase data for Ionis's Waylivra in the ultra-rare metabolic disorder familial partial lipodystrophy (FPL). The results showcased an impressive 88% reduction in triglyceride levels in the 40 participants and a 52% reduction in liver fat, confirming Waylivra's impressive lipid-lowering credentials. But given the antisense treatment's history the apparent lack of serious safety signals was also welcome. It should, however, be remembered that the results are only preliminary, and investors will rightly want a much deeper dive into any adverse events, in particular decreases in platelets. In August 2018 the FDA slapped Waylivra with a complete response letter in the related, and equally rare, familial chylomicronaemia syndrome (FCS) over clotting concerns. Interestingly, European regulators have taken a more lenient stance on the drug, which is approved in Europe for FCS, meaning that Ionis's Akcea subsidiary might focus its efforts in FPL on gaining a label extension for Waylivra in this region. However, the real prize remains the US, where the group's focus remains approval in FCS. If the benign safety profile in FPL holds up to increased scrutiny, it can only strengthen Akcea's refiling efforts.