

日本核酸医薬学会第5回年会

# Early Morning Lecture

演者

Senior vice president of development, Ionis Pharmaceuticals

**Richard S. Geary, PhD**

演題

Update of Late-stage ASO Development  
by IONIS and Recent Approvals (Inotersen)

日時

2019年7月11日(木) 7:30AM ~ 8:20AM

会場

星雲 (ホテル阪急エキスポパーク2F)



## 演題

# Update of Late-stage ASO Development by IONIS and Recent Approvals (Inotersen)

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**Richard S. Geary, PhD**

The IONIS pipeline is strategic, innovative, broad and mature. Three 2' MOE modified ASOs have achieved global market approvals over the last three years. Further, the innovations in the chemistry of systemically administered ASOs have rapidly moved into the IONIS clinical stage pipeline. Two of fourteen of the GalNAc chemistries are moving into Phase 3 development this year (APO(a) and TTR conjugated 2nd generation ASOs) along with two CNS targeted ASOs (both RNAseH1 2nd generations ASOs) for a total of four Phase 3 programs . Advances include less frequent, well tolerated administration schedules together with substantially increased safety margins. As an example of recently approved products, inotersen (Tegsedi) was approved in US, Europe and Canada in 2018, with additional country filings submitted in 2018/2019. Inotersen targets both mutant and wild type transthyretin (TTR) mRNA in hereditary TTR amyloidosis. The disease and the registration data that supported inotersen approvals will be detailed. Inotersen follow-on innovation that includes a GalNAc conjugated form, is now moving into late-stage development with promise of improved efficacy and safety apparent in early stage clinical testing. A total of ten programs are poised to enter Phase 3 in the next 2 years, including the four detailed for 2019, providing insight into the relatively near-term late stage promise of RNA targeted ASO technology.