

Betagenon/Baltic Bio announces positive results from a 28 day Phase IIa trial of the first-in-class AMPK activator O304 in Type 2 Diabetics

O304 reduces significantly fasting plasma glucose (FPG) in type 2 diabetics

The main study results are not yet finalized. Review of the initial data showed that a number of patients who were included based on HbA1c at screening, had FPG below 7 mM or above 13.3 mM at day 1, and that the data analysis of FPG required a post hoc analysis plan.

In type 2 diabetic patients on Metformin with FPG >7 mM and <13.3 mM (>126 and <240 mg/dl) at day 1, where FPG >13.3mM defines uncontrolled hyperglycemia, there were statistical significant differences between the treatment groups in the absolute and relative change in FPG at day 28. The mean absolute reduction in the Placebo group (n=24) was -0.10 mM and for the O304 group (n=25) -0.60 mM with a p-value of 0.0096. The p-value for the relative change was 0.0178. Within the treatment groups O304, but not Placebo, caused a significant absolute (p=0.0002) and relative (p=0.0003) reduction in FPG at day 28 compared to day 1.

Within the treatment groups O304, but not Placebo, caused a significant absolute reduction in HOMA-IR (p=0.0097), however, the comparison to Placebo did not reach statistical significance.

At day 40, 12 days after end of treatment, compared to day 28 there was a statistically significant difference between the groups with an absolute increase in FPG, and a relative increase in HOMA-IR in the O304 group.

O304 reduces blood pressure and increases vascular microvascular perfusion

Within the treatment groups of type 2 diabetic patients who completed examination by magnetic resonance imaging at screening and at day 28 and irrespective of FPG at baseline, O304, but not Placebo, showed a statistically significant absolute and relative reduction in diastolic blood pressure and a relative reduction in systolic blood pressure. O304, but not Placebo, also caused a statistically significant absolute and relative increase in level of peripheral microvascular perfusion, and a relative increase in the rate of perfusion.

Composite endpoint; Systolic blood pressure and peripheral microvascular perfusion

The composite endpoint is defined as a decrease in blood pressure and increase in vascular perfusion in order to be a success, all other outcomes is classified as failures. This composite endpoint showed statistically significant difference between the treatments with more successes in the O304 group, i.e. more patients had both a decrease in blood pressure and an increase in vascular perfusion.

O304 is safe

During the Phase IIa trial no safety signals were observed and O304 was well tolerated.



Thomas Edlund, CEO Betagenon AB;

“We are very pleased that the beneficial metabolic and vascular effects observed with O304 in animal disease models translated to type 2 diabetic patients in the 28 day Phase IIa trial, providing strong support for further clinical development according to plan of first-in-class AMPK activator O304 in type 2 diabetes”.

AMP activated protein kinase (AMPK) is the master regulator of energy balance. When activated by energy shortage imposed by exercise and/or caloric restriction, AMPK restores energy balance by suppressing energy consuming processes, by increasing glucose and lipid metabolism, and by increasing blood flow to deliver nutrients to target tissues. Type 2 diabetes (T2D) is characterized by both metabolic and cardiovascular dysfunction and is increasing at an epidemic rate globally. Pharmacological activation of AMPK is therefore a novel promising approach to prevent/cure T2D and associated cardiovascular disease. However, no direct AMPK activator has yet reached the clinic.

O304 is a first-in-class AMPK activator. In animal disease models O304 both increases muscle glucose uptake and reduces metabolic β -cell stress to impose β -cell rest, effects not observed by any drug currently used to treat T2D. O304 also mitigates diet-induced obesity by increasing energy expenditure, increases peripheral blood flow, and improves cardiac function and endurance in animals.

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