

# Drug Candidate for Treating Advanced Stage Neuroblastoma

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## Challenge

Pediatric neuroblastoma in its advanced stage (stage IV) is usually lethal. 70% of the affected children die. 50% of the children show upon diagnosis metastasis or a genetic amplification of the onco-gene N-myc. This group has a poor prognosis and a 5-year survival rate of only 33%. A drawback of the current standard therapy is the poor efficacy accompanied with several side effects.

## Technology

Researchers at DKFZ have identified a compound for the treatment of neuroblastomas eliciting antitumoral effects in nano-molar concentrations via its capability of inducing differentiation, growth arrest and apoptosis (Fig.1). Since normal, non-transformed cells are not affected by the compound the success rate of the therapy is improved and side effects are reduced in opposite of the current standard therapy. The anti-proliferative effect that is based apparently on inhibition of histone deacetylation is particularly strong upon N-myc amplified advanced stage neuroblastoma.

## Commercial Opportunity

We are seeking a licensing and/or a collaboration partner to further the development of this technology into a beneficial product. In particular, we are seeking an industrial partner for further development of the compound.

## Developmental Status

HC-toxin modulates a functionally critical pathway in NB cells and thereby represents a drug candidate, which is already effective at a nanomolar level, but can be further optimized. In addition, future studies identified another target which also can be developed for further drug development for treatment of advanced stage neuroblastoma. Here *in vivo* data are already available demonstrating encouraging results with high efficacy, but on the other hand low toxicity and side effects.

## Patent Situation

DKFZ filed 3 different patent families, which are pending; the first patent family is published under WO 2007/071625.

## Further Reading

Histone deacetylase inhibitor *Helminthosporium carbonum* (HC)-toxin suppresses the malignant phenotype of neuroblastoma cells. *Int J Cancer*. 2007 Dec 11; by Deubzer HE, Ehemann V, Westermann F, Heinrich R, Mechttersheimer G, Kulozik AE, Schwab M, Witt O.

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