

July 2012

# MDF News Update



MYOTONIC  
DYSTROPHY  
FOUNDATION

## Antisense - A New Treatment for Myotonic Dystrophy?

One of the most exciting areas of research in the field of muscular dystrophy - including myotonic dystrophy - is in the potential of antisense drugs. Antisense drugs are synthetic forms of RNA (the "messenger" proteins that carry genetic instructions to a cell's DNA). These drugs bind to the RNA, blocking its ability to transfer mutated genetic instructions to the DNA, thus short-circuiting a cell's ability to produce aberrant, disease-causing proteins.

Now, Isis Pharmaceuticals and Biogen Idec have teamed up to develop and commercialize just such a drug for myotonic dystrophy type 1. The drug, still in preclinical trials, is designed to fix the underlying genetic defect that causes the disease. You can read more about it [here](#).

## Isis Pharmaceuticals Presents First-Ever Pharma Research Update at 2012 Conference

MDF is excited to announce that Rob MacLeod, Vice President of Oncology and Exploratory Discovery at Isis Pharmaceuticals, will present an update on Isis's DM research work at the 2012 MDF Annual Conference on Saturday, August 18. Isis recently announced a major partnership with Biogen Idec (see above story) in the pursuit of DM treatments, and Dr. MacLeod will provide a detailed overview of the technology behind the treatments they are pursuing in partnership with the University of Rochester, how they are using the technology to approach DM, progress they have achieved in pursuit of a human drug, and the anticipated timeline for human trials. Click [here](#) to register for the conference and view the full conference program.

## Soaring Beyond DM: Kaliana and the Opera Singer

There aren't many 5 year olds who fall in love with a blind, Italian opera singer. But then, Kaliana Warford, Kali for short, was no ordinary 5 year old. Kali, who was diagnosed with congenital myotonic dystrophy at age 3, comes from a music-loving family, and she became so enamored of opera and the singer that she changed her Make-a-Wish Foundation request from visiting the Statue of Liberty to meeting and singing with opera great Andrea Bocelli. Click [here](#) to read about her remarkable journey.

## MDF 2012 Annual Conference Program Update

The MDF 2012 Annual Conference program is complete and has been updated at [myotonic.org](http://myotonic.org). This year's program is a particularly exciting and informative mix of presentations on daily living and DM management information, clinical research updates and strategies for managing complex insurance systems such as SSI and SSDI. Click [here](#) to view the final conference program, learn more about the conference location and to register.

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