New applications of CRISPR-Cas9 genetic engineering for the study of vertebrate development and disease

The advent of new approaches to manipulate vertebrate genomes is changing the realm of possibilities for studying development and disease. CRISPR, or Clustered Regularly Interspaced Short Palindromic Repeats, is a simple molecular tool that has allowed investigators to accurately edit the genome of many organisms. The method has revolutionized the fields of molecular genetics, developmental and cell biology, and regenerative medicine to name a few. CRISPR, which involves a specific Cas9 enzyme was originally discovered as part of the natural defense mechanism that bacteria have against invading viruses. Investigators have exploited this form of bacterial immunity and reported that special guide RNAs could be made that allow Cas9 to cut precisely any site in a genome. Although the cell attempts to repair the break by reattaching the two DNA strands, the inadvertent addition or deletion of DNA bases create mutations that result in the disruption of that gene. This RNA-guided Cas9 system can also repair mutations in genomes, thereby creating a way to correct genetic diseases, improve food crops and/or eradicate plant and animal pests. Recent examples of research projects into mammalian and fish research in the Western community will be used to illustrate questions and challenges in research with an emphasis on how CRISPR/Cas9-based approaches are available to accelerate research.