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Exploiting microRNA Specificity and Selectivity: Paving a Sustainable Path Towards Precision Medicine

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Abstract

In his State of the Union address before both chambers of the US Congress, President Barack Obama called for increased investment in US infrastructure and research and announced the launch of a new Precision Medicine Initiative, aiming to accelerate biomedical discovery. Due to their well-established selectivity and specificity, microRNAs can represent a useful tool, both in diagnosis and therapy, in forging the path towards the achievement of precision medicine. This introductory chapter represents a guide for the Reader in examining the functional roles of microRNAs in the most diverse aspects of clinical practice, which will be explored in this third volume of the microRNA trilogy.

Keywords

miRNA; Pharmacogenomics; Precision medicine; Initiative; Selectivity; Specificity; Pharmacogenetics

In his last State of the Union address before both chambers of the US Congress, President Barack Obama called for increased investment in US infrastructure and research and announced the launch of an innovative *Precision Medicine Initiative*. “I want the country that eliminated polio and mapped the human genome to lead a new era of medicine—one that delivers the right treatment at the right time,” he said. “Tonight, I’m launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes—and to give all of us access to the personalized information we need to keep ourselves and our families healthier,” he continued [1].

Such an announcement offers an illustration of the considerable interest that exists in achieving greater progress in treating disease [2].

President Obama has long expressed a strong conviction that research offers great potential for improving health [3]. One million or more US citizens will be powering President Barack Obama’s Precision Medicine Initiative. This bold volunteer- driven move to collect and link genotypic, phenotypic, and lifestyle data, including crowd sourcing and social media tools, aims to accelerate biomedical discovery with an initial focus on cancer [4].

The patient-participant cohort at the core of the initiative will enable new approaches to prevention, diagnosis, and treatments tailored to individual patients. “It’s a new model for doing medical research,” says National Institutes of Health’s (NIH) director Francis Collins, while discussing the precision medicine approach [3, 4].

Do microRNAs (miRs) have a role in precision medicine? The answer is yes, and apparently not only at an interindividual level but also at an intercellular level. Indeed, miRs are exquisite regulators of gene expression that inhibit translation and/ or promote mRNA degradation by base pairing to precise complementary sequences within the 3'-untranslated region.

They are expressed in a cell-specific manner and give us the possibility to generate selective treatments that target the bad cells and preserve the good cells, with major implication in cancer (see the second volume of the trilogy, where these aspects are discussed in detail) but also in other disorders [5–14].

This introductory chapter opens the third volume, where miRs will be analyzed in the clinical scenario. In the next years, clinicians will have to deal with miRs, not just as diagnostic biomarkers but also as potential tools to design selective treatments, alongside with their emerging important role in prognostic signatures and prediction models.

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