ALS (amyotrophic lateral sclerosis): A neurodegenerative disease affecting predominantly upper and lower motor neurons; also referred to as motor neuron disease. ALS is characterized by rapidly progressive muscle weakness and atrophy due to the loss of motor neurons. The most typical onset is during middle age, and most of the patients die 2-3 years after onset. More than 90% of patients have no familial history.

Cytoplasm: The contents of a cell except for the nucleus. Most chemical reactions in a cell, including translation, occur in the cytoplasm.

Dementia: A condition in which a person loses the ability to think, remember, learn, make decisions, and solve problems. Alzheimer’s disease, for instance, is characterized by progressive dementia.

FTLD (frontotemporal lobar degeneration): A disease characterized clinically by the progressive dementia due to atrophy in the frontal and temporal lobes of the brain predominantly with heterogeneous etiology.

Neurodegenerative disease: A progressive disease due to a loss of neurons with, in most cases, unknown etiology. Alzheimer’s and Parkinson’s diseases, FTLD, and ALS are neurogenerative diseases.

Nucleotides: Molecules that, when joined together, make up the structural units of DNA and RNA. The four bases found in DNA are A (adenine), T (thymine), G (guanine), and C (cytosine), and these four bases are attached to a sugar/phosphate to form nucleotides. The four bases found in RNA are A, G, C and U (uracil) instead of T.

Nucleus: The structure in a cell that contains the chromosomes (genomic DNA). DNA replication and transcription from DNA to RNA are representative reactions that take place in the nucleus.

Messenger RNA (mRNA): Protein-coding RNA that encodes and carries information from DNA to sites of protein synthesis to undergo translation. The mRNA bears the 5´ m7G cap structure (a specially altered nucleotide) and the 3´ poly(A) tail (a long sequence of adenine nucleotides), both of which contribute to effective translation and mRNA stability.
microRNA (miRNA): A class of small RNAs, ranging from 18 to 23 nucleotides in length. Approximately 2,000 human miRNAs have been identified and numbered in the order they were found (i.e.; miR-376). miRNAs are generated from long transcripts, termed primary miRNAs (pri-miRNAs), that are cleaved by the nuclear Drosha-DGCR8 complex into intermediate precursor miRNAs (pre-miRNAs), transported to the cytoplasm where they are further cleaved by the Dicer-TRBP complex, and then released as miRNA duplexes. miRNA duplexes are incorporated into Argonaute (Ago) family proteins, from which one of the two strands of the duplex is discarded, and finally the effector complex, called RNA-induced silencing complexes (RISCs) is formed. RISCs recognize complementary sites in the 3’ UTR of their target mRNAs, and induce translational repression and/or poly(A) shortening and decay of these mRNAs.

RNA-binding proteins: Proteins that bind to RNA directly through regions that can bind to RNA. This protein family is involved in RNA regulation, such as splicing. Drosha, DGCR8, Dicer, TRBP and TDP-43 are all RNA-binding proteins.

ncRNAs (non-coding RNAs): RNAs that do not encode information for translation into proteins. NcRNAs are roughly divided into two classes: long ncRNAs and small RNAs. Although the functions of most of ncRNAs remain unknown, some ncRNAs are known to participate in the regulation of gene expression. Small RNAs include miRNAs, small interfering RNAs (siRNAs) and Piwi-interacting RNAs (piRNAs). These small RNAs range from 18 to 30 nucleotides in length and modulate diverse cellular pathways.

Translation: The process of synthesizing an amino acid sequence to generate a protein product from the code in mRNA. This process takes place in the cytoplasm.

Transcription: The process by which a cell makes an RNA copy of a sequence of DNA. This process takes place in the nucleus and results in a transcript, which is RNA produced by transcription.

UTR (untranslated region): a sequence that is not translated into protein in mRNA. The 5’ side of a coding sequence is called 5’ UTR and the 3’ side is called 3’UTR. miRNAs generally bind to the 3’ UTR of mRNA.