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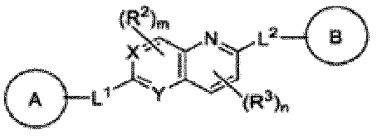
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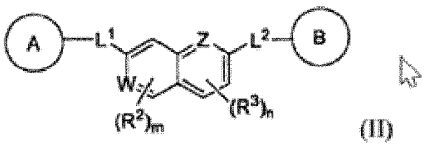
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(54) Title: 2-(INDAZOL-5-YL)-6-(PIPERIDIN-4-YL)-1,7-NAPHTHYRIDINE DERIVATIVES AND RELATED COMPOUNDS AS MODULATORS FOR SPLICING NUCLEIC ACIDS AND FOR THE TREATMENT OF PROLIFERATIVE DISEASES



(I-a)



(II)

(57) Abstract: The present disclosure features compounds of formula (I-a) and of formula (II) and pharmaceutical compositions thereof. The present disclosure further discloses the compounds of formula (I- a) and (II) and their compositions for use in methods of modulating nucleic acid splicing, e.g., splicing of a pre-mRNA, as well as said compounds for use in methods of treating e.g.: • proliferative diseases, such as e.g. cancer, benign neoplasms or angiogenesis, • neurological diseases or disorders, such as e.g. Huntington's disease, • autoimmune diseases or disorders, immunodeficiency diseases or disorders, lysosomal storage disease or disorder, cardiovascular diseases or disorders, metabolic diseases or disorders, respiratory diseases or disorders, renal diseases or disorders, or infectious diseases. Exemplary compounds are e.g. 2-(indazol-5-yl)-6-(piperidin-4-yl)-1,7- naphthyridine derivatives and similar compounds.

WO 2022/006550 A1

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TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, KM, ML, MR, NE, SN, TD, TG).

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— *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

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