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Symposium 8
Novel Agents on the Horizon for Dyslipidemia

2016年7月15日(金) 9:00-11:00　　第1会場 | 南館 5階 エミネンス

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英語

英語セッション

S8-1

MTP Inhibitor and Recent Advances in the Treatment of Homozygous Familial Hypercholesterolemia

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Homo-FH reveal limited responses to LDLR-dependent treatment like statins, and prognosis is poor without intensive treatments. LDL-apheresis is absolute indication, but a considerable burden as biweekly or weekly performed throughout their life. Liver transplantation is not feasible for many cases. There is an urgent need for novel therapy.Rare genetic diseases often give us great hint: Abetalipoproteinemia shows extremely "low LDL-C" because of VLDL-assembling-MTP deficiency. Now MTP inhibitor lomitapide has been approved for homo-FH in Western countries.Lomitapide does not require LDL receptor function, and reduces LDL-C 50% even in homo-FH if tolerated. Main adverse effects of lomitapide can be anticipated from abetalipoproteinemia. Gastrointestinal symptoms like diarrhea and fatty liver are frequent, and restriction in fat and alcohol intake is required. Also deficiency in lipid-soluble nutrients should be cared, but avoidable with supplementation. Phase III trial in Japan showed lomitapide is generally tolerable with appropriate caution for predictable adverse effects.Lomitapide should be a new feasible option for homo-FH. Recently released PCSK9 antibodies showed moderate LDL-C reduction in some part of homo-FH, even though not effective for null type. APOB antisense mipomersen has been approved in the US. These new drugs will improve the life of patients with homo-FH.